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2nd International Conference on Clinical Research Networks for Rare Diseases

9-10 December 2025

Mobilising the Global Rare Disease Clinical Research Ecosystem

Speakers

Opening Session – Setting the scene

David Pearce

David A Pearce is the chair of the consortium assembly for the International Rare Diseases Research Consortium (IRDiRC) representing funding agencies, companies, and patient advocate groups from approximately 80 countries. He is also a board member for the Undiagnosed Disease Network International (UDNI) He has published over 120 research papers on Batten disease and founded an international registry for rare diseases known as CoRDS to enable clinical trials for rare diseases.

Alexandra Heumber Perry

Alexandra Heumber Perry is the Chief Executive Officer of Rare Diseases International, the global alliance of Persons Living with a Rare Disease.

She has dedicated her entire career to contributing to improving healthcare policies to benefit people living with diseases, with a particular focus on neglected and vulnerable people.She has over 20 years of experience in global health with demonstrated capabilities in patient advocacy and multi-stakeholder partnership.

As CEO of RDI, Ms. Heumber Perry sets the strategic direction, drives the organization's goals of raising awareness of rare diseases, improving access to diagnostics, treatment and care, advocating for the rights of patients around the world and representing its members and enhancing their capacities to ultimately improve lives of persons living with a rare disease.

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IRDiRC International Rare Diseases Research Consortium
RARE DISEASES INTERNATIONAL
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Speakers

Baptiste Eluard

Baptiste Eluard has a PhD in Cancer Biology from the University of Paris, where he spent six years in academic research exploring NF-kappaB signaling as a driver of cancer aggressiveness, identifying related biomarkers, and applying multi-omic analyses. After a brief role at Home Biosciences as Portfolio Analyst, he joined the biotech startup MNEMO Therapeutics as Target Discovery Scientist, where he coordinated multidisciplinary R&D teams and implemented innovative discovery strategies to translate proteogenomics data into actionable therapeutic opportunities. Since 2024, Dr. Eluard has been Senior Scientific Project Manager at INSERM, within the ERDERA Coordination Team.

Plenary session 1: Global efforts in Real World Evidence (RWE) and data collection

Franz Schaefer

MD, Professor of Pediatrics Head, Division of Pediatric Nephrology, Heidelberg University Coordinator, European Reference Network for Rare Kidney Disease (ERKNet) Co-Lead, Clinical Research Network of the European Rare Disease Research Alliance (ERDERA)

Carla Jonker

Carla Jonker has obtained a master's degree in biomedical sciences at the University of Leiden. Currently she combines her position as National Expert in the Real-World Evidence department within the Data Analytics and Methodology Task Force at the European Medicine Agency with her work as Senior Regulatory Project Leader at the Medicine Evaluation Board in the Netherlands. She has over 20 years' experience, both in industry in different positions and at the Medicine Evaluation Board. Her work experience includes multiple topics related to the benefit-risk assessment of medicinal products and PhD research at the University of Utrecht to investigate the value of rare disease registries for regulatory decision-making.

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Speakers

Ramona Walls

Ramona Walls, Ph.D., is the Direct of the Data Collaboration Center at the Critical Path Institute, where she has worked for five years. She leads a team of Data Managers, Engineers, and Scientist that provide data products, tools, and services to make data easier to find, understand, and reuse. Before joining C-Path, Ramona led a research group at the University of Arizona's Bio5 Institute focused on semantic data integration and data infrastructure primarily in ecology, evolution, and biodiversity informatics.

Thomas Klockgether

Prof. Klockgether studied medicine at the University of Göttingen and during this time also carried out research at the Max Planck Institute for Experimental Medicine. After graduating, he went to Oldenburg for clinical training and then returned to the Max Planck Institute to work in basic research on Parkinson's disease. He completed his neurology training in Tübingen, where he also began to focus on degenerative ataxias, in addition to pursuing research on Parkinson's disease. In 1998, he was appointed as Professor and Chair of Neurology at the University of Bonn. Prof. Klockgether has been the Dean of the Medical Faculty of the University of Bonn from 2008 to 2011, and the Director of Clinical Research at the German Center for Neurodegenerative Diseases (DZNE) from 2011 to 2024. Prof. Klockgether led the team that developed and validated the Scale for the Assessment and Rating of Ataxias (SARA). He has been the coordinator of the European EUROSCA, RISCA and ESMI cohorts und one of the principal investigators of the international READISCA cohort. In 2018, he founded SCA Global which later developed into the Ataxia Global Initiative (AGI).

Pat Furlong

President, Parent Project Muscular Dystrophy

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Speakers

Plenary session 2: New approaches in diagnostics & clinical research

Yanis Mimouni

Yanis Mimouni is Associate Director for Regulatory Science at Critical Path Institute (C-Path), where he coordinates EU-funded projects and advances regulatory science. Before joining C-Path in 2025, he was Deputy Coordinator of the European Rare Diseases Research Alliance (ERDERA), a ten-year Horizon Europe Partnership of more than 170 organisations across 37 countries, overseeing the Clinical Research Network and cross-cutting regulatory and data services. He is also involved in IHI and European Commission projects such as RealiseD and INVENTS, focusing on innovative trial methodologies, in silico frameworks and patient-centred approaches. Yanis holds a PharmD and an MSc in Pharmaceutical Medicine from Université Claude Bernard Lyon 1, along with postgraduate diplomas in clinical trial evaluation, paediatric drug development and orphan drugs, as well as certifications in data science, and is based in Paris.

Alexander Hoischen

Alexander Hoischen is a genomic innovator whose research group, Genomic Technologies for Immune-mediated and Infectious Diseases at Radboud University Medical Center, has helped pioneer advanced sequencing technologies for rare diseases—especially rare immune disorders. He has been instrumental in developing novel genomic approaches, from early adoption of exome sequencing to groundbreaking work in long-read genome sequencing and optical genome mapping. Under his leadership, Radboudumc became one of the first institutions worldwide to implement long-read genome sequencing as a first-tier diagnostic assay across all rare diseases. His current research centers on immunology and rare immune-mediated and infectious diseases, with a major focus on uncovering new Inborn Errors of Immunity. His work contributed to identifying the X-linked TLR7 deficiency associated with severe COVID-19. Hoischen plays key roles in major international rare-disease programs, including leading work packages in Solve-RD (H2020) and ERDERA (2024–2031). He is the recipient of a ZonMW VICI grant supporting innovative immune-disease research and serves as Chief Innovation Officer for the Departments of Human Genetics at Radboudumc and Maastricht UMC+, as well as Scientific Director of the Radboudumc Genomics Technology Center.

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Speakers

Tudor Groza

Tudor Groza is a Principal Scientist at the Bioinformatics Institute, A*STAR, the co-lead of AI and Data Science at the Maternal and Child Health Research Institute, KK Women's and Children's Hospital, Singapore and the Deputy CTO (Health System Integration) of the National Precision Medicine Program Singapore. From July 2025, Tudor is also a member of IRDiRC Interdisciplinary Scientific Committee for a mandate of 3 years. His work spans across various dimensions of the research - clinical care continuum, from devising algorithms to support clinical decision-making, to standardisation of clinical terminology and integration with national public and private health systems. Over the course of the last 15 years, Tudor focused on contributing to the clinical phenotyping community by building deep phenotyping tools to aid the decision-making process in clinical genomics and primary care.

Sergi Aguiló Castillo

Sergi Aguiló Castillo is a Data Steward working to make Rare Disease data more interoperable and reusable through the FAIR principles. He focuses on FAIRification, semantic modelling, and building infrastructures to improve data quality. Sergi contributes to international projects such as ERDERA and JARDIN and has played an active role in numerous ELIXIR and GA4GH initiatives, helping to drive standards in health data. Based in Utrecht, he works at RadboudUMC alongside an incredible team of Rare Disease data experts.

Cécile Ollivier

Cécile Ollivier is the Vice President of Global Affairs and Managing Director for the European Office at C-Path. She is a senior health engineer with over 18 years of experience in global drug development, with a strong focus on paediatric and rare diseases. Before joining the Critical Path Institute, she worked in a medical technology company developing digital endpoints, and spent 11 years as a scientific officer at the European Medicines Agency (EMA).

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Speakers

Dustin O'Dell

Dustin O'Dell is a serial entrepreneur and data strategist with deep experience building and scaling technology companies. He began his career at Experian before joining AdTheorent as its sixth employee, helping drive the company from zero to $120M in annual revenue and a $1B IPO in 2021. He also co-founded Barometric, a division of AdTheorent that was acquired 18 months after launch. Most recently, Dustin co-founded SymetryML, the parent company of Decentra Health. As CEO of Decentra Health, he is focused on building healthcare's global collaboration layer—bringing real-time, privacy-preserving intelligence to rare disease research worldwide.

Christian Hendriksz

Christian Hendriksz serves as Chief Community Impact Officer at A Rare Cause, a registered charity in England and Wales dedicated to advancing rare disease services in low- and middle-income countries. He is also Chief Medical Officer at Decentra Health, a pioneering data collaboration company leveraging patented AI technology to transform healthcare insights. At IRDiRC, Christian Hendriksz serves as a member of the Diagnostics Scientific Committee (DSC) since December 2024, with a mandate of 3 years.

Dr. Yong Chen

Dr. Yong Chen is a Professor of Biostatistics and Professor of Biomedical Informatics at the University of Pennsylvania. His research focuses on clinical evidence generation, machine learning and artificial intelligence, and the development of learning health systems. At Penn, he founded the PennCIL (Penn Computing, Inference, and Learning) Lab and established a Type I Center, the Center for Health AI and Synthesis of Evidence (CHASE).Dr. Chen is one of twenty Commissioners serving internationally on the Lancet Commission on Rare Diseases. Dr. Chen serves as a Statistical Editor for the Annals of Internal Medicine, a Statistical Consultant for The New England Journal of Medicine – AI, and an Associate Editor for both JASA and The Annals of Applied Statistics. Dr. Chen has published 300 papers, including more than 100 papers on clinical journals. He is an elected Fellow of the American Statistical Association (ASA) and the American College of Medical Informatics (ACMI).

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Speakers

PARALLEL WORKSHOP 1: Learnings from Low and Middle-Income Countries: Ensuring Representation in Clinical Research Data and Registries

Daria Julkowska

Daria Julkowska has a PhD in molecular biology and pursued her scientific vocation by the post-doctoral experience in cellular biology, at Institut Pasteur, Paris and extensive training in communication and European Union counselling. She also holds MSc in Management of Research from the University of Paris Dauphine. She coordinated the European Joint Programme on Rare Diseases and is currently the scientific coordinator of ERDERA rare diseases partnership that brings together over 180 institutions representing different type of stakeholders (researchers, funders, clinicians, industry & patients) from 37 countries from Europe and beyond. She is involved in the rare diseases field since 2010. She developed and put into action a set of collaborations facilitating research, including the partnerships with European Research Infrastructures, Patients' Organizations and industry. She has an extensive knowledge and understanding of European funding schemes and programmes and served as the chair of the Expert Group on support for the strategic coordinating process for European partnerships of the European Commission.In 2020 she received EURORDIS Black Pearls Award for the European Rare Diseases Leadership. In 2024 she obtained the title of "Remarkable Pole" in France, and Prix INSERM in the category of Research Support for her achievements and continuous support of research in the rare diseases field.

Monica McClain Drum

Dr. Monica McClain Drum is an epidemiologist with extensive experience in rare diseases. She has held positions in academia, where her research involved ways to improve access to, and quality of care, for people living with rare diseases and in industry where she focused on health outcomes and epidemiology of rare diseases, primarily using real-world evidence. Currently, Dr. Drum works in Global Programmes at RDI and is involved in projects that seek to improve access to diagnosis, healthcare and treatment.

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Speakers

Chris Hendriksz

A Rare Cause (United Kingdom / South Africa)

Roberto Giugliani

Roberto Giugliani, Full Professor at the Postgraduate Program of Genetics and Molecular Biology at Federal University of Rio Grande do Sul, is a medical geneticist who coordinates the Clinical Research Group in Medical Genetics of Hospital de Clínicas de Porto Alegre, in Brazil. He is Head of Rare Diseases at Dasa Genomics and Executive Director of Casa dos Raros, a center entirely dedicated to the diagnosis, care, and training in rare diseases. He is also the Editor-in-Chief of the Journal of Inborn Errors of Metabolism and Screening, Member of the Brazilian Academy of Sciences, and co-chair of the Rare Diseases International-Lancet Commission in Rare Diseases.

Mohamed Hassany

Mohamed Hassany serves as the Health Minister's Assistant for Projects and Public Health Initiatives at the Ministry of Health and Population (MoHP) in Egypt. He is also an Associate Professor of Infectious Diseases and Hepatogastroenterology at the National Hepatology and Tropical Medicine Research Institute (NHTMRI) in Cairo. Additionally, he holds the position of Executive Director of the National Committee for Control of Viral Hepatitis (NCCVH), MoHP, Egypt. Dr. Hassany's expertise and dedication were acknowledged when he was appointed as a member of the Regional Validation Committee (RVC) for the Elimination of Mother-to-Child Transmission (EMTCT) and the control and elimination of hepatitis B and C within the WHO Eastern Mediterranean Regional Office (EMRO) In recognition of his leadership in healthcare, Dr. Hassany was chosen as a member of the Governing Board of the African Medicine Agency (AMA) of the African Union. He is also a member of the Governing Board of the Pandemic Fund. In recognition of his leadership in healthcare, Dr. Hassany was chosen as a member of the Governing Board of the African Medicine Agency (AMA) of the African Union. He is also a member of the Governing Board of the Pandemic Fund. Furthermore, Dr. Hassany is a Board Member of the Access to COVID-19 Tools (ACT) Accelerator Council of the WHO and served as a board member on the Regional Advisory Panel for the MENAT (Middle East, North Africa, Afghanistan, and Pakistan) Initiative of the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, USA.

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Speakers

Robin Sarfati

CTO at Tekkare

PARALLEL WORKSHOP 2: Rules of engagement in multi-stakeholder pre-competitive environments

Samantha Parker

Samantha Parker is Patient Advocacy Lead at Italfarmaco and Vice Chair of the International Rare Disease Research Consortium. She has over two and a half decades of international rare disease experience in the biopharmaceutical industry. She has a proven track record in including the patient voice in research, small molecules, gene therapy development, natural history studies, registries, novel patient-centred outcomes, healthcare education and collaborative networks. At IRDiRC, Samantha is currently involved in task forces set up to better understand the complexity of funding for rare disease research and motivating factors for organisations to invest. Samantha is an adamant believer that patients should be at the front and centre of rare disease research and development. She has been among the thought leaders of patient-academic-industry collaboration from the early 2000s which led her to become involved in policy making for rare diseases and she served on the EU committee of experts in rare diseases. She was involved in determining the policy framework for the establishment of European Reference Networks for Rare Diseases.

Heidrun Hildebrand

Alliance Manager Pediatric Development; Bayer Pharmaceuticals

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Speakers

Ricardo Fernandes

Ricardo Fernandes is an Associate Professor in Clinical Pharmacology, a Consultant Pediatrician, and a Clinical Researcher, with a research background in clinical epidemiology, clinical trial methodology and operations, pediatric clinical pharmacology, and synthesis research. He has collaborated in the inception and ongoing activities of pediatrics research collaborations and networks across different disciplines, and served in learned societies and research organizations. He has led the establishment of a national pediatric trial network, STAND4Kids Portugal, and assumed the role of Work Package and National Hub lead in the conect4children/c4c IMI/IHI consortium that created a paneuropean clinical trial network. Ricardo Fernandes is a founding Board Member of the conect4children-Stichting (Foundation), where he serves as Chief Medical Officer since its incorporation in 2023.

Cécile Ollivier

Vice President Global Affairs, Critical Path Institute (The Netherlands)

Begonya Nafria Escalera

Begonya Nafria, Head of Patient Engagement in Research at Sant Joan de Déu Chidren's Hospital (Spain). She has long experience in the field of the involvement of patients and families in research initiatives. She has also a personal story as a cancer patient, and as a caregiver and patient advocate because is the sister of a young adult with cerebral palsy. She is currently a PhD student in the patient involvement in paediatrics field, specifically in a thesis project about the rights of children and young people participating in clinical trials. Fellow of EUPATI (first cohort) and member of its Academic Cluster, Coordinator and Founder Member of eYPAGnet (European Young Patients Advisory Group Network, and member of the Patients and Families working group and Chair of the Cross-Border Access to Paedaitric Clinical Trials of EnprEMA (European Network of Paediatric Research of EMA).

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Speakers

Volker Straub

Professor Volker Straub is the Director of the John Walton Muscular Dystrophy Research Centre and Deputy Dean for Newcastle University's Translational and Clinical Research Institute. He has a long-standing interest in disease mechanisms of genetic muscle diseases, with activities involving diagnostics and clinical trials. He was the co-founder of the EU FP6 funded network of excellence for genetic neuromuscular diseases, TREAT-NMD, and is involved in many large EU supported translational research projects. Volker represents the UK on the Scientific Committee of the European Cooperation in Science and Technology (COST).

Kristina an Haack

MD, Senior Project Head Rare Development, Inherited NeuroMetabolic Diseases, Pediatric Network Lead

Victoria Hedley

Rare Disease Policy Manager and Co-Lead of the Newcastle University Centre of Research Excellence in Rare Disease - Together4Rare

Plenary session 3: Reporting back from parallel workshops & discussion

Monica McClain Drum

Senior Global Programmes Manager, Rare Diseases International (France)

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Speakers

Daria Julkowska Scientific coordinator of the European Rare Diseases Research Alliance (ERDERA) and Assistant Director of the Thematic Institute for Genetics, Genomics & Bioinformatics (IT GGB) at INSERM (France)

Heidrun Hildebrand Alliance Manager Pediatric Development; Bayer Pharmaceuticals

Plenary session 4: Global trials, local impact: Empowering access and engagement in international clinical research, role of patients and broad partnerships

Alexandra Heumber Perry Chief Executive Officer, Rare Diseases International (France)

Dr Sudheendra Rao Scientific Advisor, Organization for Rare Diseases India

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Speakers

Christine Mutena

Christine Mutena is a passionate advocate for rare diseases and disability in Kenya and globally. As the co-founder of Rare Disorders Kenya (RDK), she amplifies the needs of the rare disease community through awareness and policy advocacy. A mother of two children with rare genetic conditions, she is dedicated to ensuring children with disabilities live full, independent lives with dignity.

Begonya Nafria Escalera

Head – Patient Engagement in Research Area, Pediatric Cancer Center Barcelona, Sant Joan de Deu SJD, Spain; Conect4Children Stichting

Roberto Giugliani

Professor of Genetics at the Federal University of Rio Grande do Sul (UFRGS), Chief of the Clinical Research Group in Clinical Genetics at Hospital de Clinicas de Porto Alegre (HCPA), and Executive Director of Casa dos Raros, in Porto Alegre (Brazil)

Plenary session 5: Global networks and models of care in high income and LMIC – The Duchenne experience

David Pearce

International Rare Diseases Research Consortium (IRDiRC) Professor Department Pediatrics, Sanford School of Medicine of the University of South Dakota (United States of America)

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Speakers

Anna Thetford

Anna Thetford is a Senior Registered Nurse and integral member of the Rare Care Centre based in Perth, Western Australia. Anna has a commitment to advocating for patients with disability and complex needs and for the past 20 years. She has been instrumental in developing and leading paediatric services with a focus on creating high performance multidisciplinary teams to deliver excellence in clinical care. Anna completed a Churchill Fellowship in 2025 exploring Family Support Programs for Rare Disease to further enhance her ability to impact patient care positively. Anna has a commitment to ensure consumer co design is incorporated in all new service development.

Karolína Podolská

Dr. Karolina Podolska has been involved with rare diseases, particularly neuromuscular diseases, for over ten years. As an internal physician based in the Czech Republic, she started a transition program for patients with neuromuscular diseases coming from pediatric to adult clinic and she has been the coordinator of the Center for adults with neuromuscular diseases in General Faculty Hospital in Prague since 2023. She is actively involved in European and Czech projects engineering new changes in the health care system for RD patients and serves as a board member in the World Duchenne Organization (WDO) and Parent Project Czech Republic. She is the Program Manager of the Accredited Duchenne Centers Program, developed by WDO, where she works alongside experienced Duchenne care experts to support centers that seek not only accreditation but also practical guidance on how to further enhance the quality of their care. Thanks to this program and to her own experience with applying for accreditation, she will present how accreditation can serve as a practical framework for improving Duchenne care quality across diverse health systems.

Ricardo Fernandes

Chief Medical Officer at conect4children-stichting

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Speakers

Karolina Śledzińska

MD, PhD, Pediatrician, Clinical Geneticist, Department of Pediatrics, Hematology and Oncology, Medical University of Gdansk, Poland

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