2nd International Conference
on Clinical Research Networks

CONNECTED FOR IMPACT

December 9-10, 2025
Heidelberg, Germany

EVENT REPORT

ERDERA
European Rare Diseases Research Alliance

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

RARE DISEASES INTERNATIONAL

Co-funded by the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595. Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.

INTRODUCTION

The **second international clinical research network (CRN) conference** was held on 9-10 December 2025 at Marsilius College, Heidelberg, Germany. It was co-hosted by **European Rare Diseases Research Alliance (ERDERA), International Rare Diseases Research Consortium (IRDiRC) and Rare Diseases International (RDI)**. There were approximately 90 in-person speakers and attendees and 370 online registrants. **These participants represented 77 countries, of which half were non-European. Most classified themselves as researchers (51%), followed by patients/advocates (28%) and clinicians (23%)**.

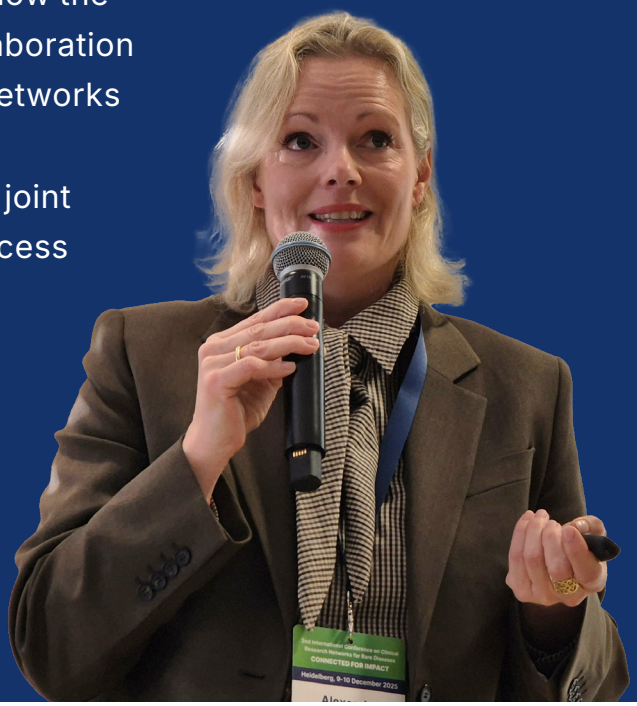
This conference built on the 2022 international conference of the CRNs on rare diseases in Paris, France, which focused on increasing mutual knowledge on CRN's structure and activities and identify routes to stimulate cooperation and interoperability of these networks on a global scale. The objective of this second conference edition was to identify ways to enable participation of low- and middle-income countries (LMICs) in clinical research and registries by assessing current gaps and learning from their experiences, successes and challenges, and developing strategies for their inclusion in CRNs, with the goal to reduce geographical inequities and to maximize data collection.

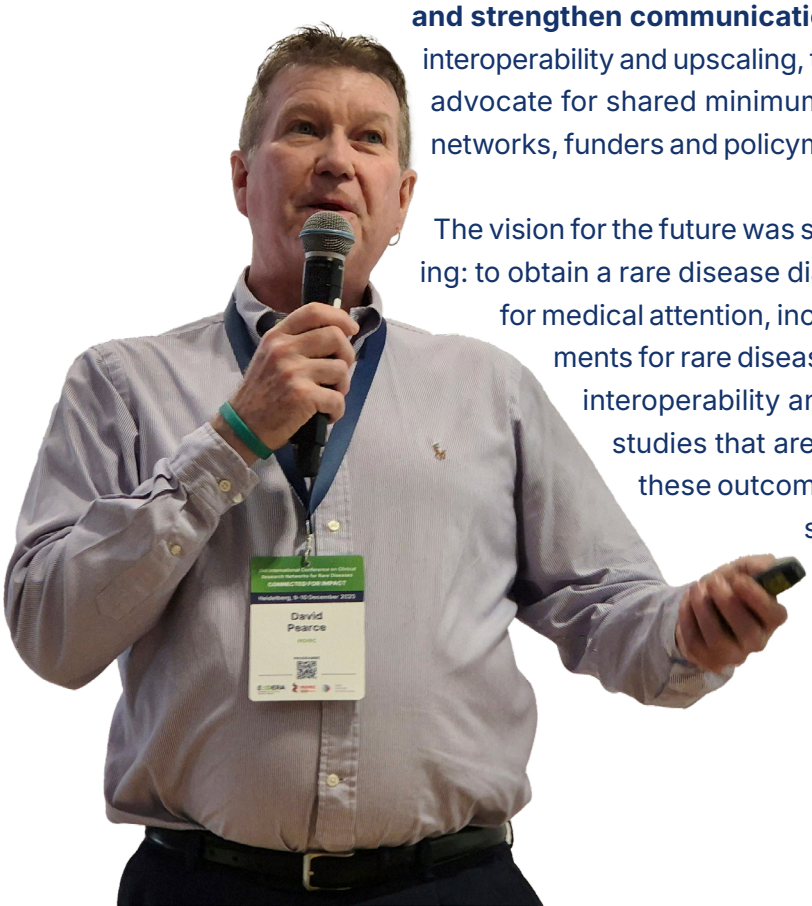
DAY
1

OPENING SESSION

SETTING THE SCENE

The conference started with a **'past, present, future' perspective to depict the continuity of CRN activities**. The past was summarized by IRDiRC on how the previous conference focused on the collaboration and interoperability of clinical research networks and how they should optimally function: connecting on a global scale, developing joint roadmaps for improved collection and access to standardized data, sharing expert knowledge and benefiting from input and experiences of people living with rare diseases (PLWRD). The topic of 'where are we now' was presented through the lens of PLWRD by RDI.





Key points included: **bring PLWRD priorities into CRN collaborations and decision-making, support inclusive engagement across geographies and strengthen communication and transparency**. In order to achieve interoperability and upscaling, the following recommendations were made: advocate for shared minimum datasets and outcomes, connect across networks, funders and policymakers and coordinate among conferences.

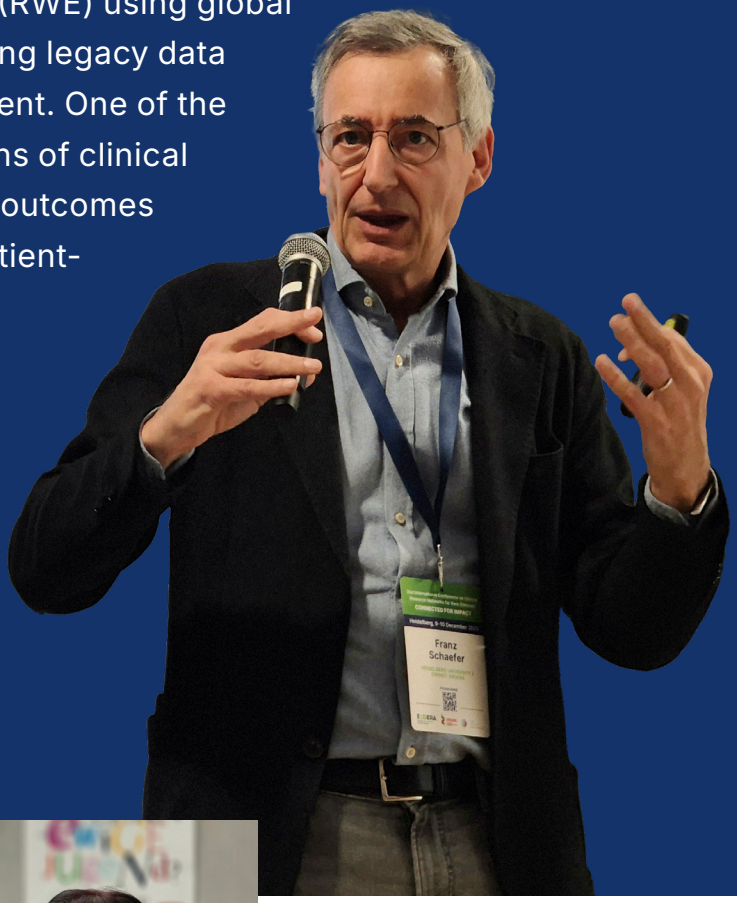
The vision for the future was shared by ERDERA as key objectives including: to obtain a rare disease diagnosis within six months after presenting for medical attention, increasing the development of effective treatments for rare diseases, development of cross-CRN priorities in interoperability and the implementation of two to three pilot studies that are co-led by CRNs. To successfully achieve these outcomes, the perspectives of PLWRD are necessary, there should be lightweight governance and the roles of PLWRD and industry must clearly be defined.

DAY
1

PLENARY SESSION 1

GLOBAL EFFORTS IN REAL-WORLD EVIDENCE AND DATA COLLECTION

This session provided examples from international efforts in data collection and sharing, harmonization of outcome measures and biomarkers and a platform that turns real-world data (RWD) into real-world evidence (RWE) using global coordination and partners, harmonizing legacy data and creating tools for drug development. One of the presentations discussed the limitations of clinical trial data and the need for real-world outcomes that are collected in registries and patient-reported outcomes. The European Medicines Agency perspectives on the generation of RWE for regulatory purposes and how RWD supports decision-making were shared.



DAY
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PLENARY SESSION 2

NEW APPROACHES IN DIAGNOSTICS AND CLINICAL RESEARCH

This session presented a **continuum of how rare diseases are detected, characterized, and addressed through research and care**. It opened with emerging methods for automated extraction of structured data from electronic health records (EHRs), enabling the real-time capture of clinically relevant phenotypic information at scale.

These advances help generate richer diagnostic contexts and feed directly into downstream applications, including phenotype-genotype correlation and AI-driven case identification. Building on this, the session highlighted Next-Generation Diagnostics designed to maximize diagnostic yield and reduce time-to-diagnosis. These tools combine long-read genomic sequencing and multi-omics, with clinically guided interpretation to improve diagnostic precision —especially for patients with atypical or unsolved conditions. Crucially, this knowledge must translate into therapeutic solutions.

The final presentation explored **advances in Drug Development Tools (DDTs)** —including extrapolation tools, modular disease models, validated biomarkers and Integrated Disease Models— that support more efficient and patientcentered clinical trial designs and Precision Care.

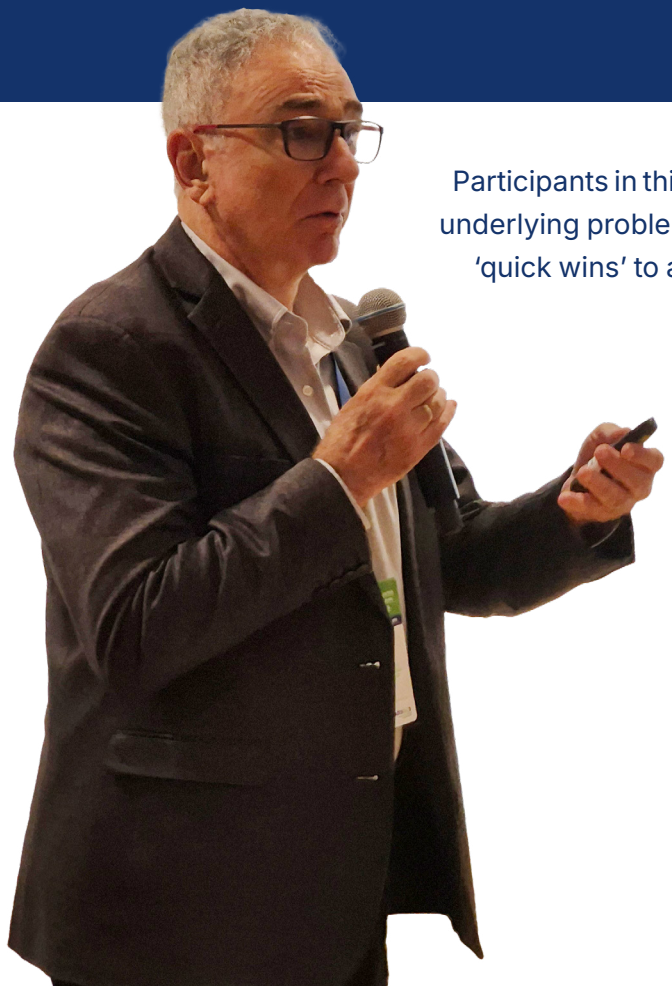
These tools are essential to reducing development timelines and regulatory barriers, bringing treatments closer to patients. Finally, the session emphasized that innovation must be inclusive. Approaches must be tailored to resource-limited settings —such as privacy-preserving decentralized AI and scalable, open-source diagnostics— that ensure these breakthroughs benefit underserved populations and enable global participation in research.

DAY
2

PARALLEL WORKSHOP 1

LEARNINGS FROM LOW- AND MIDDLE- INCOME COUNTRIES: ENSURING REPRESENTATION IN CLINICAL RESEARCH DATA AND REGISTRIES

This workshop began with **four short presentations** that included an overview of the newborn screening (NBS) program in Egypt, how Brazil developed a model to obtain access to high-cost advanced therapies, an application for health care providers (HCPs) to assist in the diagnosis of rare diseases and existing clinical research and registry initiatives in LMICs.



Participants in this workshop were divided into four groups to identify underlying problems within the topics of the four short presentations, 'quick wins' to address them and possible long-term actions.

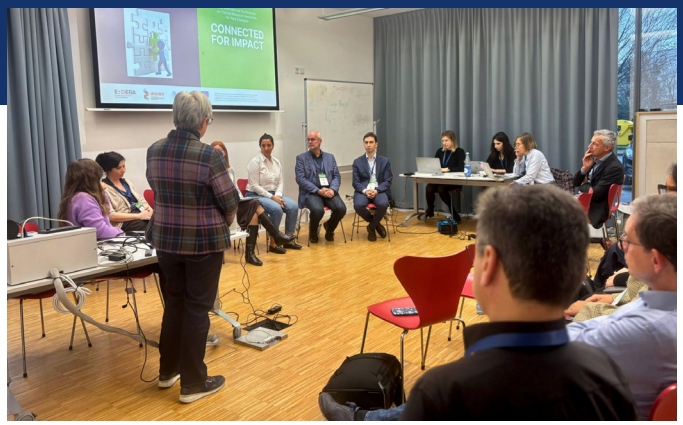
The results of this workshop included practical solutions to underlying barriers and challenges that are ready to be implemented (see Roadmap)

DAY
2

PARALLEL WORKSHOP 2

RULES OF ENGAGEMENT IN MULTI-STAKEHOLDER PRE-COMPETITIVE ENVIRONMENTS

This workshop also began with **four presentations with examples of multistakeholder projects** pertaining to improving pediatric clinical trial design and delivery, developing drug development tools to inform regulatory decision-making, overcoming fragmentation in neuromuscular disease research, care and clinical trials and enabling responsible collaboration between European Reference Networks and industry.



Presentations were followed by a **panel discussion** around the following themes: patient engagement, sustainability versus unmet medical need, governance and legal structures, data sharing, trust and readiness and the role of neutral, pre-competitive spaces.

> **The results from this workshop call for cultural and relational collaboration, in addition to technical, long-term programmatic projects and deeper connection among patient engagement, data governance and sustainability**

DAY
2

PLENARY SESSION 3
REPORTING BACK FROM PARALLEL WORKSHOPS & DISCUSSION

The summaries from the two parallel workshops were presented and discussed. See Roadmap for further details.

PLENARY SESSION 4
GLOBAL TRIALS, LOCAL IMPACT: EMPOWERING ACCESS AND ENGAGEMENT IN INTERNATIONAL CLINICAL RESEARCH, ROLE OF PATIENTS AND BROAD PARTNERSHIPS

This session was a **moderated panel discussion** in the question-answer format to explore policy frameworks that support international clinical research, highlight how patients and communities drive equity and innovation, examine models and opportunities for partnerships and global access to trials and identify key levers for global-local alignment. Participants included a patient advocate from Kenya, a clinical researcher from Spain, a geneticist from Brazil and a physician-scientist from India. The questions focused on patient-centeredness in involving LMICs in research and registries.

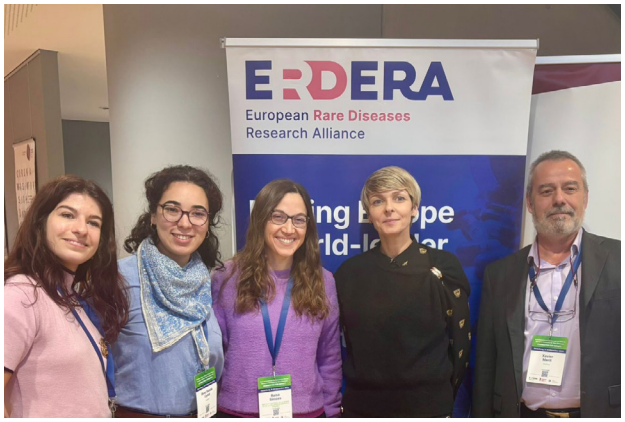
DAY
2

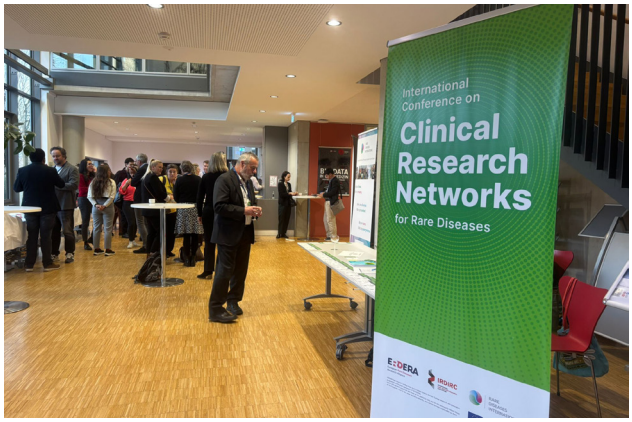
PLENARY SESSION 5

GLOBAL NETWORKS AND MODELS OF CARE IN HIGH-INCOME AND LMIC —THE DUCHENNE EXPERIENCE

To support individuals living with rare and undiagnosed diseases globally, purposeful care and services across the patient journey for all age groups is imperative. These pathways must incorporate strategies for early diagnosis, timely therapeutic interventions, coordinated care delivery and psychosocial support.

This session included **presentations** on the optimization of the clinical aspects of care models for PLWRD, the role of accreditation in the quality of care for PLWRD and the importance and benefits of holistic care coordination for rare diseases support.





SUMMARY ROADMAP

The roadmap for strengthening global rare disease CRNs revolves around several strategic objectives mirroring the themes of the conference.

First, **collaboration needs to be strengthened within a global, interoperable CRN ecosystem.**

Secondly, there is a need for **improving RWE production and high-quality data infrastructure** (e.g. enabling use of registry and EHR data)

Third, **accelerating diagnostic innovation will require identifying CRNs capable of deploying AI-driven phenotyping and long-read sequencing.**

Fourth, **all these goals will require the expansion of LMIC participation and capacity-building,** so that regional research hubs can be created in the longer term.

Next, **innovation in clinical trial design and execution, including through greater involvement of patients, is crucial, while the establishment of sustainable, multi-stakeholder governance** —following the blueprint of CPath and C4C among others— **will be central to sustaining the work done in CRNs.**

Finally, **focusing on the quality of life and improving care pathways for patients and families according to recognized models** (e.g. Rare Care Centre in Australia) **will be a priority.**

INTERNATIONAL CRN DETAILED ROADMAP OF ACTIVITIES TO IMPLEMENT BY 2027

The roadmap will be operationalized according to the **seven strategic objectives detailed in the summary above and culminating in an eighth task that consolidates these strategic goals.**

The most immediate task, to be achieved within six months, will be to **publish a map of existing CRNs to facilitate global collaboration.** This will be the launchpad for European Reference Networks (ERNs) to pilot automatic data extraction from Electronic Health Records (EHR) systems, and for ERDERA diagnostics research leaders to pilot long-read genome sequencing. LMIC participation can be strengthened in the short term with local awareness campaigns, potentially led by patient advocates and local governments.

Longer term actions, in the interest of achieving the strategic goals by the next International CRN Conference in late 2027, **will require the engagement of a variety of stakeholders, including patient advocates, clinicians, researchers, policymakers, regulators, industry and clinical research teams.**

The activities detailed in the roadmap **should be complementary to, and build off, existing work** being conducted within the **Global Commission to End Diagnostic Odyssey for Children with a Rare Disease** and the commitments espoused by **WHO Member States that unanimously adopted the Seventy-eighth World Health Assembly Resolution on Rare Diseases in 2025** ("Rare diseases: a global health priority for equity and inclusion").

PLANNED ROADMAP ACTIVITIES

1. **Publish a map of existing CRNs** to identify complementarities and gaps and thus facilitate collaboration among existing networks in Europe, LMICs and beyond. The global CRN collaboration map should also allow for the establishment of a CRN match-making mechanism, by which researchers could pair their networks to launch joint pilots or other forms of collaboration.

2. **Agree on data harmonization and interoperability among CRNs.** To enable the kind of global collaboration described above, the definition and adoption of a minimum common dataset, or a shared interoperability standard, for all CRNs will be an essential next step. Adopting such standards will also require alignment on a common data quality requirement, for example EMA or RDCA-DAP frameworks for regulatory-grade RWE. Pilots can be launched to test the use of AI for the automatic extraction of common data elements from EHR systems.

3. **Select diagnostic pilot sites,** namely by identifying CRNs ready to test AI-assisted phenotyping and long-read sequencing, that will allow for the launch of 2-3 CRN co-led pilots that also focus on interoperability. Results from these pilots can be integrated into CRN clinical pathways.

4. **Increase LMIC participation.** In the short term, this could be done by establishing national patient hubs, launching country-level awareness campaigns and micro-pilots in newborn screening and registry development. Another short-term goal is to increase access to the Rare Disease Knowledge (RDK) application, including the development of an offline version of the app for use in remote areas with limited Internet access. In the longer term, strengthening national governance, policy alignment, training and registry connectivity will be important to create regional research hubs.

5 **Implement more inclusive, patient-led clinical trials that will increase access to trials.** This will involve integrating patient co-creation requirements early on, starting with trial design. The aim should be to deploy decentralized trials, via satellite sites and telehealth, and to address barriers that undermine cross-border access to trials (e.g. visa restrictions, ethics approvals). Ultimately, the adoption of a global "minimum trial readiness" standards for ethics, data interoperability, regulatory pathways and patient-reported outcome (PRO) validation methods will streamline the implementation of clinical trials across all CRNs.

6 **Develop sustainable and transparent multi–stakeholder governance,** based on the lessons of C-Path, C4C, TREAT-NMD and Together4RD. A first step will be to establish a neutral CRN Coordination Secretariat, which will serve as an independent facilitator to manage collaborations, legal templates, data-sharing and multi-year programmes. Long-term strategic planning is necessary from the outset and will require sustainability mechanisms that address funding sources (public and private) and potential revenue models.

7 **Improve care pathways and quality of life for people living with rare disease.** Select models, such as accredited care centers for Duchenne and the Rare Care Centre in Australia, can provide blueprints for deploying multidisciplinary integrated care pilots at scale addressing mental health and social support in addition to diagnostics and primary care. International quality-of-care standards should be established to harmonize standards for accreditation, patient satisfaction measures, emergency pathways- among other elements – across the global CRN.

8 **Ultimately, aim to expand diagnostic and interoperability pilots, launch policy recommendations to international bodies (e.g. WHO, EMA) and publish common KPIs** for assessing diagnostic timelines, trial recruitment diversity and data quality and interoperability.

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