

RARE DISEASE RESEARCH UK

NEWSLETTER

A forum to illuminate the latest advancements within the rare disease research community.

Rare Disease Research UK (RDR UK) is a £14 million platform to advance research into rare diseases. Jointly funded by MRC and NIHR, RDR UK is made of 11 research nodes - involved in both disease-area-specific and cross-cutting research, engaging leading universities across the country - and a coordinating hub, hosted by Newcastle University, Newcastle upon Tyne Hospitals NHS Foundation Trust and Genetic Alliance UK.

As a UK-wide platform, we hope to be able to significantly impact the rare disease research landscape and improve the lives of those directly or indirectly affected by rare diseases.

We're thrilled to share the third edition of our quarterly newsletters, packed with insights, activities and updates from across the RDR UK Platform that we're eager to share with you.

With each edition, we aim to illuminate the latest advancements within the rare disease community, share compelling stories, and foster collaboration among researchers, industries, and patients.

HIGHLIGHTS

Annual in-person Platform Executive Board (PEB) Meeting - January 2025



The meeting was held on 28th January 2025 at UCL, London, bringing together members from the Hub, wider Node teams, and external speakers from the MRC, Rare Therapies Launchpad, and the Oxford-Harrington Centre.

HIGHLIGHTS

2nd Annual RDR UK Conference - March 2025



The 2nd Annual RDR UK Conference took place on 25th March at Hyatt Regency, Manchester, bringing together industry, patient groups, healthcare professionals and researchers for a day of insightful discussions and collaboration. The theme of the conference, 'Demystifying the Landscape', was aimed at unravelling the various network groups and their significant contributions to rare disease research. With 109 attendees present in-person, 30 joining virtually, and exhibitors from PSPA, Genomics England, Cure DHDDS and UNIQUE, the conference served as a vital opportunity to explore the landscape of rare disease research and the substantial progress being made in the field.

[Find out more about the day..](#)

HIGHLIGHTS

Early Career Researcher (ECR) Award in Public and Patient Involvement and Engagement (PPIE)

We were delighted that our inaugural PPIE Award for Early Career Researchers in Rare Disease attracted a really strong pool of applicants, representing research as diverse as co-designing a national patient day which generated new research ideas, embedding patient voice in a clinical trial platform and co-producing a scoping review and peer-reviewed paper on adult social care. What each of the most highly scoring submissions had in common was a real partnership approach between researchers and people living with rare conditions, and an emphasis on working toward shared goals and real impacts.

CONGRATULATIONS !!

Winners: Dr Polly Livermore, Nathanael Bourns & Kaya Bourns
Runners up: Dr Akshay Deshpande & David Hulme



Find out more and take a look at some insightful blogs from our applicants.

Follow the link below if you missed our past event!

Webinar on Introduction to Co-Production

What does co-production really mean in rare disease research, and how does it work in practice? This webinar explores the role of patient advocacy groups in shaping meaningful research and what effective collaboration looks like for both researchers and contributors.

Check out the recording

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UK Platform for Nucleic Acid Therapies - UPNAT

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Ethical Legal and Social Issues in Rare Conditions Research and Clinical Practice - ELSI

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Renal Ciliopathies National Network - CILIAREN

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Rare Early Onset Lower Urinary Tract Disorders - REOLUT

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mTOR Pathway Diseases

Rare Disease Research UK.

Cardiovascular Initiative

Rare Disease Research UK.

Lipidomics and Metabolomics for Rare Disease Diagnosis

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Epigenomics of Rare Disorders - EpiGenRare

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Parkinson Plus Syndromes - ExPRESS

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HistioNode: Histiocytic Neoplasms and HLH

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Changing Clinical Practice Through Innovative Trial Designs - CAPTIVATE

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