

# Rare Disease Clinical Trial Network

## HRB Rare Disease Research and Innovation Catalyst Awards



Prof Rachel Crowley

Consultant Endocrinologist SVUH / Clinical Professor University College Dublin

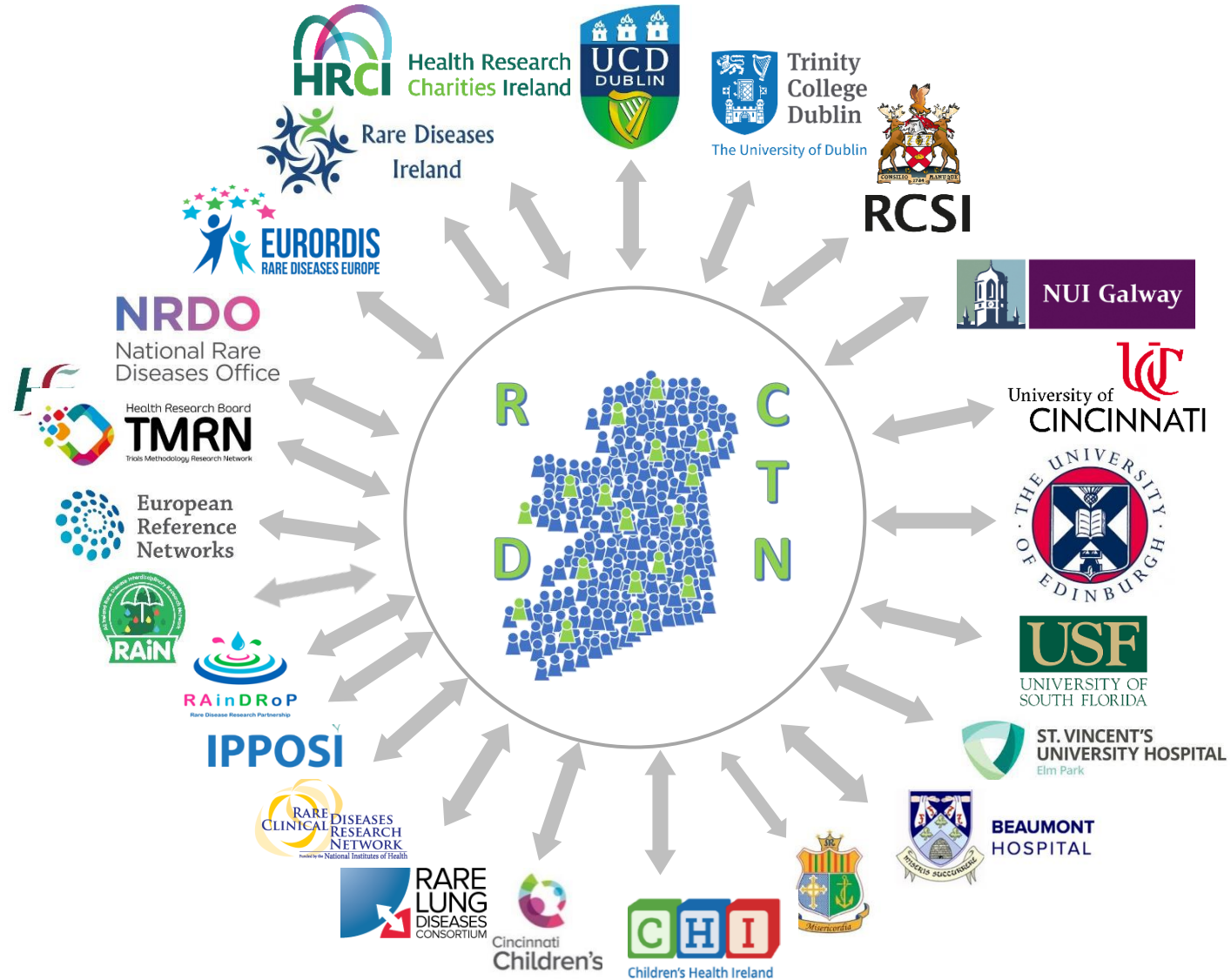
Co-lead Rare Disease Clinical Trial Network

Adult lead Irish ERN BOND centre

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# Rare Diseases Clinical Trial Network





# Access to Rare Disease Clinical Trials in Ireland

- 2015 - 2020: 47 trials in RD (excluding cancers) at Irish sites; 17 in cystic fibrosis
- Only 2 academic investigator-led studies
- Estimated 300,000 people in Ireland living with rare disease
- 2022, 18 European Reference Network centres for clinical care rare disease approved in Ireland



# Work Packages of RD CTN

- Application
- Prioritisation
- Support provided

Governance

Data

- Database - patients with RD interested in research
- Workshop consent & data access
- FAIR data policy
- Template portfolio

- PPI Liaison; PAG
- Social media presence
- PPI PROM
- Core Outcome Set selection
- Conference
- Representation at international meetings
- RD CTN publications
- Contribution to policy
- Access to treatments

PPI & Impact

Methodology & Training

- Methodology support
- Design
- HRB TMRN partnership
- Workshops trial methodology & statistical analysis
- Adaptation UCD education RD (patient) investigator needs
- SWAT & qualitative research



# Where is the RDCTN at now? WP1

- Network Manager Brendan Molloy started Oct 2022 email [brendan.molloy1@ucd.ie](mailto:brendan.molloy1@ucd.ie)
- PPI Liaison officer shortlisting week 20/2/23
- DIFA application 2022, follow on with industry
- Network expansion – RAIN; NIRDRWG
- Supporting NRDO on research inquiries
- Supporting NCTO on feasibility queries (6 RD in 4 fields)
- Advisory group meeting 5/12/22
- Weekly operational group meetings
- SOPs inc network expansion, privacy reviewed by Advisory Group
- Consultancy agreements reviewed by UCD legal department
- Ongoing contacts re RD trials, presentations, liaison with patient groups & pharmaceutical industry, grant applications



## Where is the RDCTN at now? WP2

- Mailing list for RD interest being built mailchimp; patient input received
- Database industry partners including CROs
- Portfolio of template trial documents
- Registered with EU Networks / matchmaking tools
- Meeting with Cystinosis academic researchers re supports for trials in Ireland



# Where is the RDCTN at now? WP3

Rare diseases require clinical innovation — being open to all possibilities

'You can't ever hang your hat on just one thing ... because you don't know what's going to work'

- Recruitment of Patient Advisory Group members ongoing
- Website development ongoing; using NCTO (Juvo)
- Patient input on PAG, DIFA, mail list, website, methodology meeting
- Irish Times RDCTN profile article, with patient interview
- UCD PPI Ignite Involvement podcast (Ep5)
- Meeting with RDI re PCOM work Dec 22
- Network Manager attended EURORDIS round table of companies meeting Brussels 22/2/23
- Presenting patient meetings Feb & April 2023, including outcome QoL in OI publication Orph J Rare Dis
- Rare Disease conference planning 29<sup>th</sup> Feb & 1<sup>st</sup> March 2024




## Where is the RDCTN at now? WP4


- Application for EJPRD networking support scheme for webinar July 2023  
*Building networks and expertise in rare disease research methodology*  
(in collaboration with HRB TMRN, Cystinosis Ireland, methodologists Gottingen Germany, Nijmegen Netherlands & Vienna Austria)
- Support request ERA NET Neuron Networking Group call 2022 Chronic Pain
- Impact – invitation XLH international guidance development Dec 2022 (AXLES & BUR02 trials); also progressing reimbursement for burosumab in adults with XLH
- Early career researchers' journal club, invite to first speaker
- Supporting ICT Day event UCD 2023 with training webinar







# How could RDCTN contribute to RD Partnership?

- SO1: Patient-need led relevant research enabled by outcome-oriented investments strategically deployed along the R&D value chain 
- SO2: Better understanding of RD burden and impact assessment of interventions.
- SO3: Interoperable, federated, evolving and scalable RD infrastructure of data, samples, resources and tools with necessary critical mass for meaningful RD research & innovation.
- SO4: Active utilisation in all Member States & Associated countries by all stakeholders of high-value, ethically and regulatory compliant data tools and services tailored to needs of RD research community.
- SO5: Decreased number of unsolvable cases as well as reduction in the duration of the diagnostic odyssey.

SO6: Increased capacity of RD stakeholders across Europe through quality training and skills development. 

- SO7: Reduced failure rates of therapeutic development
- SO8: Meaningful empowerment, engagement, and leadership - as equal partners - of people living with a rare disease. 
- SO9: Effective alignment of national RD strategies with EU objectives, leveraging shared resources and maximising MS and Associated countries contributions.
- SO10: Effective transcontinental collaboration. 

Co-ordination of Irish submissions  
Application support  
Stakeholder engagement