Rare Disease Research Conference, 5th November 2026, UCD

Registration	Tea and Coffee	**08.20**
Welcome	Prof Rachel Crowley and Prof Cormac McCarthy Co-leads RDCTN	**08.50**

Session 1

Chairs
Ms Liz Molloy
Dr Emer Doheny

Orphan Medical Devices - Innovation and Regulation — **09.00**

09.00 **Regulatory Perspective** – Dr Donal O'Connor, HPRA
09.10 **Industry Perspective** – Ms Gwynne Morley, IQVIA & HealthTech Ireland
09.20 **Orphan Devices Key Advances** – Dr Anneliene Jonker, University of Twente
09.35 **Panel Discussion**

Chair
Dr Marissa O'Callaghan

Trial Spotlights — **09.55**

09.55 **EB Shield -** Dr Fiona Browne, Children's Health Ireland
10.05 **PROSERA study** – Dr Sarah Cullivan, Mater University Hospital
10.15 **RDCTN Seed Fund Award Update -** DysK - measuring disease severity in cervical dystonia (pilot study) – Dr Owen Killian

---

PPI Leadership

Shaping and Modelling Best Practice through Partnership — **10.30**
Dr Cassy Dinius, RDCTN

Networking — ***Poster Review, Refreshments*** — ***10.45***

Session 2

Chairs
Dr Sarah Moran
Prof Jeffrey Krischer

Innovation, Regulation & Data Systems — **11.15**

11.15 **Innovative Methodologies for Rare Disease Clinical Trials** - the RealiseD Approach - Prof. Dr. Ralf-Dieter Hilgers, RealiseD
11.35 **Pathways to Rare Disease Therapies** - Prof Claire Booth, Great Ormond St Hospital & University College London
11.55 **Health & Data Systems for Research & Clinical Trials** Prof Catherine Quinlan, Children's Health Ireland
12.15 **Panel Discussion**

Chair
Prof Peter Doran

Patient Spotlight: My Clinical Trial Experience — **12.30**

Ms Linda Sheehan, IMPALA 2 Participant in conversation with **Prof Bruce Trapnell**, Cincinnati Children's Hospital (PI), **Mr Brian Robinson**, Savara.

Networking — ***Poster Review, Lunch*** — ***13.00***

RARE DISEASE CTN
RARE DISEASE CLINICAL TRIAL NETWORK

ÉIRE2026.EU
IRELAND2026.EU
Imeacht Gaolmhar | Associated Event

Rare Disease Research Conference, 5th November 2026, UCD

Research Spotlight	**Advanced Therapy Medicinal Products** - Prof Martina Hennessy, St James's CRC	**14.00**
Award	**Prof Sean Ennis Tribute & Presentation of the 2027 Sean Ennis Seed Fund Award**	**14.15**

Session 3

Chairs
Prof Bruce Trapnell
Prof Sinéad Murphy

Efficiency & Effectiveness in Treating Rare Diseases — **14.30**

14.30 **Introduction: A Patient Perspective** – Ms Eva Lally, RDCTN PPI Panel
14.45 **REMEDi4ALL – Patient-centred medicines repurposing** – Mr Anton Ussi, Eatris & Remedi4All
15.05 **Case Study: MOI-A Study** - Prof Nick Bishop, University of Sheffield
15.20 **Panel Discussion**

Networking	***Poster Review, Refreshments***	***15.30***

Chair
Prof Stuart Ralston

Poster Lightning Talks — **16.00**

16.00 **From Mutation to Degeneration:** Developing Novel Models to Understand Warburg Micro Syndrome – Ms Ailbhe Conway
16.05 **Novel variants in ATP6V0a1** are associated with dysfunction of autophagy, nutrient sensing and mitochondria – Dr Kasia Whysall
16.10 **Patient-centred care**, the patient voice and professional identity formation – our students reflect on their 'Rare Genetic Disease' learning journey – Dr Niamh Banville

Session 4

Chairs
Ms Avril Daly
Prof David Keegan

Policy Driven Innovation — **16.15**

16.15 **European Policy & Partnerships** – Dr Daria Julkowska, ERDERA
16.25 **National Rare Disease Strategy** – Prof Atif Awan, National Rare Disease Office
16.35 **Policy Driven Research Northern Ireland** – Prof AJ McKnight, Queens University Belfast
16.45 **Panel Discussion**

Awards	**Best Poster Award Presentations**	**17.00**
Meeting Close	Prof Rachel Crowley and Prof Cormac McCarthy Co-leads RDCTN	**17.10**
Drinks Reception	**Conservatory**	**17.20**