RARE DISEASE CLINICAL TRIALS CONFERENCE 2024

THURSDAY, 29 FEBRUARY

8:00 – 9:00	Registration & poster set-up (O'Carolan Room)
Main Meeting Tara Room	
9:00 – 9:15	Welcome & introduction
9:15 – 9:45	Challenges and Solutions to Conducting Clinical Research in Rare Respiratory Diseases **Prof Elisabeth Bendstrup | Aarhus University, Denmark**
9:45 – 10:15	Training the Next Generation in Rare Disease Clinical Research **Prof Debra Regier | Chief, Division of Genetics and Metabolism, Children's National Hospital, USA**
10:15 – 10:45	Perspective on Research **Lorraine McGlinchey | Ulster University**
10:45 – 11:00	Tea break (O'Carolan Room) Meet & Greet: Research Data (Marconi Room) **Sarah Forde | RDCTN** **Godfrey Fletcher | Cystic Fibrosis Registry of Ireland**
11:00 – 12:00	Panel Session 1: How to Translate Basic Research into a Clinical Trial **Chairs: Prof Peter Doran | UCD Prof Peter Conlon | RCSI** **Panel: Francis X McCormack | University of Cincinnati Adam Byrne | UCD Rachel Crowley | SVUH**
12:00 – 12:30	Clinical Trial Design Issues and Options for Study of Rare Diseases **Prof Jeffrey Krischer | University of South Florida, USA**
12:30 – 13:30	Lunch & posters (O'Carolan Room)
13:30 – 14:30	Panel Session 2: How to Involve PPI in Trial Design & Engage Patient Participants in Trials **Chairs: Dr Avril Kennan | HRCI Dr Cassandra Dinius | RDCTN** **Panel: Alessandro Franciosi | SVUH Daniel Mikula | SVUH Liam Galvin | EU-IPFF**
14:30 – 15:00	The Power of Patient Organisations in Developing Meaningful Rare Disease Therapeutics **Prof Francis X McCormack | Gordon and Helen Hughes Taylor Chair of Internal Medicine, University of Cincinnati, USA**

15:00 – 15:30 Coffee (O'Carolan Room)

15:30 – 16:00 Rare Disease Research Catalyst Consortium
Prof Rachel Crowley | Consultant Endocrinologist, St. Vincent's University Hospital, Ireland

16:00 – 17:00 Keynote: Pulmonary Macrophage Gene Therapy
Prof Bruce Trapnell | Cincinnati Children's Hospital, USA

17:00 – 18:30 Reception and poster award (O'Carolan Room)

FRIDAY, 1 MARCH

8:30 – 9:30 Registration (O'Carolan Room)

Main Meeting Tara Room

9:30 – 10:00 Gene therapy – Real World Application
Prof David Keegan| Consultant Ophthalmologist, MMUH, Dublin

10:00 – 10:30 Rare Disease Clinical Trials from The Patient Perspective/PPI
Avril Daly | CEO, Retina International & President EURORDIS

10:30 – 11:30 Panel Session 3: What outcomes are important to patients?
Chairs: Dr Suja Somanadhan | UCD_RAIN Vicky McGrath | Rare Disease Ireland
Panel: Jeffrey Krischer | University of South Florida
Bruce Trapnell | Cincinnati Children's Hospital Avril Daly | EURORDIS

11:30 – 11:45 Tea break (O'Carolan Room)

Meet & Greet: Patient and Public Involvement (Marconi Room)
Dr Cassandra Dinius | RDCTN
Dylan Keegan | UCD Clinical Research Centre

11:45 – 12:15 Achieving Regulatory Approval for Orphan Drugs
Dr Gene Sullivan | Insmed Inc.

12:15 – 12:45 Challenges in Rare Disease Clinical Trials of Related Rare Disease Phenotypes: Insights from Neurological Movement Disorders
Dr Laura Williams | Consultant Neurologist, St. Vincent's University Hospital, Ireland

12:45 – 13:00 Seed Funding Award Announcement
Prof Cormac McCarthy| UCD

13:00 – 13:15 Wrap up followed by lunch