Rare Diseases Ireland

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Imeacht Gaolmhar | Associated Event

PROGRAMME - DRAFT

Research as Care: Why Rare Diseases Matter for Europe's Future

Embedding Research and Innovation as Standard of Care in Rare Diseases

Friday 13 November 2026

Radisson Blu Royal Hotel, Dublin, Ireland

09:15-10.00	**Registration, Delegate Sign-In (Onsite and Hybrid Platform Opens)** *Tea, Coffee and Light Refreshments*

OPENING PLENARY

10:00–10:10	**Welcome and Introduction to the Day** *Vicky McGrath, Chief Executive Officer, Rare Diseases Ireland*
10:10–10:25	**Opening Address** *To be confirmed*
10:25–10:55	**Opening Plenary Session** *Framing the day: research and innovation as standard of care for the rare disease community. The opportunity for Europe to embrace rare disease innovation and drive both health and economic benefits for its citizens* Speakers • *Joanna Drake, Deputy Director-General for Research and Innovation, European Commission* • *Javier Padilla, Secretary of state for health for the Ministry of Health of Spain* • *Emer Cook, Executive Director, European Medicines Agency* • *Agnes Cser, Member, European Economic and Social Committee* • *Further Speakers TBC*

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THEMATIC SESSION 1 - The Biotech Act & Europe's Innovation Base

11:00–11:15

Scene-Setting – Developer's experience
Introductory remarks framing the session theme
Les Martin, parent and advocate

11:15–12:15

The Biotech Act and Europe's Innovation Base - *Panel discussion*
Can Europe create the conditions to turn scientific excellence into innovation, investment and patient benefit? The Biotech Act offers a chance to reduce fragmentation, improve the regulatory environment, mobilise investment and speed the journey from discovery to clinical application. The session asks what Europe needs to get right - from clinical trial pathways and regulatory sandboxes to investment, manufacturing, data, genomics and AI - so biotech research becomes an engine for growth driving competitiveness, economic growth and better health outcomes for the people of Europe.

- *Rosa Castro, Public Affairs Director, EURORDIS – Rare Diseases Europe*
- *Professor Martina Hennessy, Director of the Wellcome HRB Clinical Research Facility, St James's Hospital, Dublin*
- *Alexandre Mejat, Deputy Director, International Scientific Networks, AFM-Téléthon*
- *Stefano Romanelli, Government Affairs Associate Director, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)*
- *Muiris O'Connor, Assistant Secretary, Research and Development and Health Analytics, Department of Health, Ireland*
- *Further Panellists TBC*

12:15–13:45 **Buffet Lunch -** *Onsite, for all attendees*

THEMATIC SESSION 2 - Research & Innovation as Standard of Care

13:45–14:00

Scene-Setting - Patient's Experience
Introductory remarks framing the session theme
Mary Nicholson, Head of Advocacy, Debra Ireland

13:45–15:00

Research and Innovation as Standard of Care - *Panel discussion*
From policy to practice: how can people living with rare diseases access research and innovation as an integral part of their care, rather than as a matter of geography, chance or individual persistence? The session demonstrates what partnership looks like in practice - patients co-designing trials, registries turning routine clinical data into research, and patient voices and data informing regulatory and reimbursement decisions. Its central claim: patient partnership is not an optional addition to research, but one of the mechanisms that makes research faster, better and more relevant - and a health system built this way is a more attractive environment for clinical research, investment and talent.

- *Mary Nicholson, Head of Advocacy, Debra Ireland*
- *Godfrey Fletcher, Chief Executive Officer, Cystic Fibrosis Registry of Ireland*
- *Dr Avril Kennan, Chief Executive Officer, Health Research Charities Ireland (HRCI)*
- *Prof Sally Ann Lynch, Consultant Geneticist, Children's Health Ireland; Incoming Coordinator, ERN ITHACA; President elect, European Soc of Human Genetics (ESHG)*
- *Dr Daria Julowska, Strategy & Partnerships in Health Lead, ERDERA Scientific Coordinator*
- *Further Panellists TBC*

15:00–15:30 **Networking Break -** *Tea and coffee*

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THEMATIC SESSION 3 - The European Blueprint for Rare Diseases: An Action Plan for Europe

15:30–15:45	**Scene-Setting – Community Experience** *Introductory remarks framing the session theme* *Jean Saslawsky, Chief Executive Officer, EURORDIS-Rare Diseases Europe*
15:30–16:45	**The European Blueprint for Rare Diseases: An Action Plan for Europe - *Panel discussion*** *How do we connect and scale these opportunities across Europe? More than 30 million Europeans live with a rare disease, yet major disparities in diagnosis, care and access persist between and within countries - challenges that cross borders and cannot be solved by fragmented national approaches alone. The patient-led European Blueprint for Rare Diseases, led by EURORDIS and due for publication in October 2026, offers the foundation for a coherent EU Action Plan - one capable of connecting the Biotech Act, European research infrastructure, data and genomics, ERNs, and pharmaceutical policy so that investment in innovation translates into better health and economic outcomes for Europe.* • *Jean Saslawsky, Chief Executive Officer, EURORDIS – Rare Diseases Europe* • *Nicolas Casares, MEP, European Parliament,* • *Prof Ellen Crushell, Deputy Chief Medical Officer, Department of Health, Ireland* • *Antoni Montserrat Moliner, Vice-President, ALAN-Maladies Rares, Luxembourg* • *Agnes Cser, Member, European Economic and Social Committee* • *Dr. Seán Byrne, Associate Director, Science Policy & Regulatory Affairs, European Federation of Pharmaceutical Industries and Associations (EFPIA)* • *Further Panellists TBC*

CLOSING

16:45–17:15	**Closing Address – speaker TBC** *Closing remarks – Avril Daly, EURORDIS-Rare Diseases Europe & Rare Diseases Ireland*
17.15-18.45	**Early Evening Networking Reception**

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