PROGRAMME

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	**In Person Registration (Hybrid Platform Opens 09.45)** *Tea, Coffee and Light Refreshments*
10:00–10:10	**Welcome and introduction to the day** *Vicky McGrath, Chief Executive Officer, Rare Diseases Ireland* **Opening address** *Nicolás González Casares, MEP (Spain), European Parliament*

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

10:10–10:25

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

10:25–11:35

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.

- *Alexandre Mejat, Deputy Director, International Scientific Networks, AFM-Téléthon*
- *David Keegan, Clinical Professor of Ophthalmology and Retina, and Consultant Ophthalmic Surgeon, Mater Hospital, Dublin*
- *Fabio D'Atri, Policy Officer, European Commission (DG SANTE)*
- *Les Martin, parent and advocate*
- *Muiris O'Connor, Assistant Secretary, Research and Development and Health Analytics, Department of Health, Ireland*
- *Rosa Castro, Public Affairs Director, EURORDIS-Rare Diseases Europe*
- *Stefano Romanelli, Government Affairs Associate Director, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)*
- *Vytenis Andriukaitis, MEP (Lithuania), European Parliament Rapporteur of the Biotech Act*

In Partnership with:

THEMATIC SESSION 2 - Research & Innovation as Standard of Care

11:40–11:55

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

11:55–13:00

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.

- *Avril Kennan, Chief Executive Officer, Health Research Charities Ireland (HRCI)*
- *Daria Julkowska, Strategy & Partnerships in Health Lead, ERDERA Scientific Coordinator*
- *Godfrey Fletcher, Chief Executive Officer, Cystic Fibrosis Registry of Ireland*
- *Joanna Drake, Deputy Director-General for Research and Innovation, European Commission*
- *Juan Garcia Burgos, Head of Public and Stakeholder Engagement, European Medicines Agency*
- *Mary Nicholson, Head of Advocacy, Debra Ireland*
- *Sally Ann Lynch, Consultant Geneticist, Children's Health Ireland; Incoming Coordinator, ERN ITHACA; President elect, European Soc of Human Genetics (ESHG)*

13:00–14:00 **Buffet Lunch**

VOICES OF EUROPEAN LEADERSHIP

14:00–14:15

Welcome back and introduction
Vicky McGrath, Chief Executive Officer, Rare Diseases Ireland

Keynote address
Michael McGrath, European Commissioner for Democracy, Justice, the Rule of Law and Consumer Protection

14:15–15:00

Voices of European Leadership
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

- *Agnes Cser, Rapporteur of the European Economic and Social Committee's exploratory opinion "Leaving No One Behind: European Commitment to Tackling Rare Diseases"*
- *Barry Andrews, MEP (Ireland), European Parliament*
- *Joanna Drake, Deputy Director-General for Research and Innovation, European Commission*
- *Juan Garcia Burgos, Head of Public and Stakeholder Engagement, European Medicines Agency*
- *Nicolás González Casares, MEP (Spain), European Parliament*
- *Padraig O'Sullivan, TD, Irish Parliament*

15:00–15:30 **Networking Break**

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

15:45–17:00

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.

- *Agnes Cser, Rapporteur of the European Economic and Social Committee's exploratory opinion "Leaving No One Behind: European Commitment to Tackling Rare Diseases"*
- *Anna Rath, Orphanet Director*
- *Antoni Montserrat Moliner, Vice-President, ALAN-Maladies Rares, Luxembourg*
- *Ellen Crushell, Deputy Chief Medical Officer, Department of Health, Ireland*
- *Jean Saslawsky, Chief Executive Officer, EURORDIS-Rare Diseases Europe*
- *Seán Byrne, Associate Director, Science Policy & Regulatory Affairs, European Federation of Pharmaceutical Industries and Associations (EFPIA)*
- *Susan O'Connell, Consultant Paediatric Endocrinologist, Children's Health Ireland; Incoming Paediatric Chair and Vice-Coordinator Endo-ERN*

CLOSING

17:00–17:15

Closing Remarks
Avril Daly, President, EURORDIS-Rare Diseases Europe
Vicky McGrath, Chief Executive Officer, Rare Diseases Ireland

17.15-18.45 **Networking Reception**

Thank you to all our speakers, panellists, partners and sponsors, and to everyone in the room and online. Most of all, thank you to the rare disease community. Your experiences, insights and determination remain at the heart of everything we do. We are stronger together!

Ní neart go cur le chéile

Strength with unity

All speaker and panellist biographies are available online

The Biotech Act & Europe's Innovation Base
https://rdi.ie/biotech-act/

Research & Innovation as Standard of Care
https://rdi.ie/research-as-care/

Voices of European Leadership
https://rdi.ie/voices-eu-leadership/

The European Blueprint for Rare Diseases: An Action Plan for Europe
https://rdi.ie/blueprint-to-action-plan/

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