Advancing the Rare Brain Disease Ecosystem

Rare Disease Day 2026

Day: In the framework of Rare Disease Day, **Tuesday 24 February 2026**

Duration: 09.00 – 15.45 – Hybrid

Location: BlankSpace Place du Luxembourg, **Rue d'Arlon 80, 1040, Brussels, Belgium**

The main purpose of Rare Disease Day is to raise awareness about rare diseases and their impact on the lives of those affected. Building on the European Brain Council's (EBC) ongoing work on rare diseases, the Rare Brain Disease (RBD) Ecosystem was launched in 2025. This initiative aims to engage key stakeholders across Europe to identify gaps, set priorities, and foster collaborative projects, with a focus on strengthening the integration of research and healthcare. Placing brain health at the core of rare disease prevention and management, this event gathers multidisciplinary experts from research, clinical care, patient advocacy, and industry to share perspectives and priorities on the rare disease research, care, and policy space.

The meeting will review recent European project and regulatory developments and discuss practical strategies to advance the RBD Ecosystem and build a comprehensive Knowledge Hub. Experts will also explore how care and treatment pathways for Myasthenia Gravis (MG), Neurofibromatosis Type-1, and Childhood Dementia can be improved, drawing on both patient and clinician perspectives. Highlights include the launch of the Rethinking MG Advocacy Paper and the announcement of a new Neurofibromatosis Type 1 project.

Provisional Agenda

8.30 – 9.15 Registration & Welcome Coffee

9.15 – 9.20 Welcome & Opening

Speakers:

Sameer Zuberi, President-Elect, European Brain Council (EBC)

Kailash Bhatia, President-Elect, European Academy of Neurology (EAN)

9.20 – 9.35 The Community's Perspective

Session Type: Presentation

Objective: Presenting outcomes of the EURORDIS Rare Barometer survey and outlining initiatives from the Social Policy Action Group (SPAG) supporting EURORDIS's work to shape inclusive policies and practices for access to holistic care and uphold the social and human rights of people living with rare diseases and their families.

Speakers:

Véronique Van Assche, Member, Social Policy Action Group, Spinal Muscular Atrophy (SMA) Belgium & SMA Europe

Jessie Dubief, Social Research Director, Rare Barometer Programme Lead, EURORDIS

9.35 – 9.45 Interaction with the audience: Q&A

9.45 – 10.30 SESSION 1

Advancing the Rare Disease Agenda in Europe

Session Type: Panel Discussion

Objective: Set the stage by highlighting the impact of rare diseases on patients and families and exploring how recent EU policy & regulatory developments (pharma legislation, Biotech Act, Life Science Strategy) can accelerate research, innovation, and access.

Moderator:

Holm Graessner, Coordinator, European Reference Network for Rare Neurological Diseases (ERN-RND)

Speakers:

Daria Julkowska, Scientific Coordinator, European Rare Diseases Research Alliance (ERDERA), INSERM

Victor Maertens, Government Affairs Director, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

Hélène Le Borgne, Policy Officer, Directorate-General for Research and Innovation, European Commission

Caterina Giovagnoni, Policy Analyst, Directorate-General for Health and Food Safety, European Commission

10.30 – 10.40 Interaction with the audience: Q&A

10.40 – 11.05 Coffee Break

11.05 – 11.50 SESSION 2

Advancing the Rare Brain Disease Ecosystem and Building a Knowledge Hub

Session Type: Panel Discussion

Objective: Discuss the added value of the RBD Ecosystem & Knowledge Hub as a platform for collaboration across research, care, and policy. Engage stakeholders in defining priorities, synergies, and next steps for rare diseases. Introduce the knowledge hub and gather perspectives on the following proposed thematic areas, applying a brain health approach across rare diseases, paediatric-onset conditions and transition of care, and digitalisation and health literacy, as well as the added value of the digital platform beyond current resources.

Moderator:

Sameer Zuberi, Vice-President, European Brain Council (EBC), Past President, European Paediatric Neurology Society (EPNS)

Speakers:

Holm Graessner, Coordinator, European Reference Network for Rare Neurological Diseases (ERN-RND)

Katrin Rabiei, Chair, European Association of Neurosurgical Societies (EANS) Research Committee

Kailash Bhatia, President-Elect, European Academy of Neurology (EAN)

Astri Arnesen, President, European Federation of Neurological Associations (EFNA)

11.50 - 12.00 Interaction with the audience: Q&A

12.00 – 12.20 The European Strategy for Rare Diseases

Session Type: Keynote Address

Objective: Update on the state of play of the European Strategy for Rare Diseases.

Speaker:

MEP Tomislav Sokol, European People's Party (EPP, Croatia)

12.20 – 13.30 Networking lunch

13.30 – 14.35 SESSION 3

Rethinking Care Pathways: The Myasthenia Gravis Case Study

Session Type: Project progress presentation and panel discussion

Objective: Provide an update on the Rethinking Myasthenia Gravis (MG) project, originally launched at the 2025 event. Present key outcomes from the project's Advocacy Report, along with findings from the systematic literature review on the socio-economic burden of MG. Using this initiative as a case study, the discussion will gather multidisciplinary experts and highlight opportunities to enhance diagnosis, improve standards of care and strengthen patient involvement in treatment decision-making.

Moderator:

Vinciane Quoidbach, Research Project Manager, European Brain Council

Speakers:

Giuseppe Turchetti, Professor in Economics and Management of Innovation in Healthcare, Scuola Superiore Sant'Anna

Anne Bruijnes, Neurologist, neuromuscular disorders, Maastricht UMC+

Lorenzo Maggi, Neurologist, Fondazione IRCCS Istituto Neurologico Carlo Besta

Lutgarde Allard, President, European Myasthenia Gravis Association (EuMGA)

Lenja Wiehe, Global Patient Advocacy Director, Alexion

Anna Kole, Global Patient Engagement Lead, UCB

14.35 – 14.45 Interaction with the audience: Q&A

14.45 – 15.30 SESSION 4

Advancing Knowledge in Rare Diseases: Neurofibromatosis and Childhood Dementia

14.45-15.00: The Childhood Dementia Project Update

Session Type: Presentation followed by a video message.

Objective: Provide an update on progress and needs to promote research and development of novel treatments and improve quality of care for children with dementia, including work for reaching consensus on disease definition and EBC involvement in fostering dialogue and stakeholder engagement.

Speakers:

Vinciane Quoidbach, Research Project Manager, European Brain Council

Kristina Elvidge, Head of Scientific Affairs, Childhood Dementia Initiative (video message)

15.00 - 15.30: The Rethinking Neurofibromatosis (NF) Type 1 Project Announcement – A Cross European Study of Access to Care, Psychosocial Burden and Unmet Needs Among Patients Living with Neurofibromatosis Type 1

Session Type: Panel Discussion

Objective: Introduce the new NF-1 project, outlining key objectives and methodologies.

Moderator:

Vinciane Quoidbach, Research Project Manager, European Brain Council

Speakers:

Vera Lipkovskaya, Public Policy and Project Manager, NF Patients United

Claas Rohl, Chairman, NF Kinder, NF Patients United

Clinician, Member or Lead, Expert Advisory Group (tbc

15.30 – 15.40 Interaction with the audience: Q&A

15.40 – 15:45 Wrap Up and Closing Remarks

Speaker:

Kailash Bhatia, President-Elect, European Academy of Neurology (EAN)