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 - 16.00 – Hybrid

Location: BlankSpace 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 brain 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 brain 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 brain disease research, care, and policy space.

The meeting will focus on reviewing key developments in regulatory frameworks and discussing strategies to advance the RBD Ecosystem and develop a comprehensive Knowledge Hub. Moreover, experts will explore how care and treatment pathways for diseases such as Myasthenia Gravis (MG), Neurofibromatosis Type 1, and Childhood Dementia can be improved from both the patient and clinician perspectives. Key highlights include the launch of the Rethinking MG Advocacy Paper and the announcement of a new project on Neurofibromatosis Type 1.

KEYNOTE – The European Strategy for Rare Diseases

Speaker: ***MEP Tomislav Sokol, European People's Party, Croatia***

SESSION 1 - Panel Discussion - Advancing the Rare Brain Disease Agenda in Europe

Objective: Exploring how recent EU policy & regulatory developments (pharma legislation, HTA Regulation, 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)

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

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SESSION 2 - Panel Discussion – Advancing the Rare Brain Disease Ecosystem and Building a Knowledge Hub

Objective: Discuss the added value of the Rare Brain Disease Ecosystem & Knowledge Hub as a platform for collaboration across research, care, and policy. Engage stakeholders in defining priorities, synergies, and next steps for rare brain diseases. Introduce the knowledge hub and gather perspectives on the two proposed thematic areas, pediatric-onset rare neurological diseases and digitalization and health literacy, and 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)

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SESSION 3 - Project progress presentation and panel discussion – Rethinking Care Pathways: The Myasthenia Gravis Case Study

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.

Speakers:

Vinciane Quoidbach, Research Project Manager, European Brain Council

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

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SESSION 4 - Panel discussion – The Rethinking Neurofibromatosis type 1 project announcement – "***A Cross-European Study of Access to Care, Psychosocial Burden and Unmet Needs Among Patients Living with Neurofibromatosis Type 1"***

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)***