

Towards a Rare Brain Disease Ecosystem

Day: 29th February 2024 – Rare Disease Day

Duration: 10.00 - 14.00

Location: Francqui Room, University Foundation, Rue d’Egmont 11, 1000 Brussels, Belgium

Aim

To raise awareness on rare brain disease, this event will bring together researchers, clinicians, persons living with brain disease and industry to share their perspectives and priorities in the rare brain disease research, care, and policy space. This will result in the 2024 strategic plan for the EBC rare brain disease ecosystem.

Preliminary Agenda

Session 1: Welcome and opening

10.00 - 10.05 Welcome

Frédéric Destrebecq, European Brain Council, Executive Director

10.05 - 10.10 Opening Address

TBC

10:10 – 10:25 The MOONSHOT initiative

Magda Chlebus, Federation of Pharmaceutical Industries and Associations (EFPIA)

Session 2: Patient and caregiver priorities

10.25-10.35 A testimony

Tim Buckinx, Epihunter

10:35 – 10:45 Mental health in people living with a rare disease

Matt Bolz-Johnson, EURORDIS – Rare Diseases Europe

10.45-10.55 The rare neurology charter

Orla Galvin, European Federation of Neurological Associations (EFNA)

Session 3: Industry innovation trends

10.55-11.05 Neuromyelitis optica spectrum disorder (NMOSD) and Myasthenia Gravis (gMG)

Matthias Heck, Alexion Pharmaceuticals

11.05-11.15 Epilepsy & Rare Syndromes

TBC

11.15-11.25 Amyotrophic Lateral Sclerosis, a rare neurodegenerative disease

Lugdivine Le Dez, Amylyx Pharmaceuticals

Session 4: Clinical care pathways for rare disease in Europe

11.25-11.35 The case of ataxia

Paola Giunti, Ataxia UK

11.35-11.45 The case of phenylketonuria

Anita MacDonald, Birmingham Children's Hospital, UK

11.45-11.55 Towards a digital care pathway?

Giuseppe Turchetti, Scuola Superiore Sant'Anna, IT

11.55-12:05 Bringing Artificial Intelligence home for a better care of amyotrophic lateral sclerosis

TBC

Session 5: Open Discussion: Unmet needs

12.05-12.35 Healthcare professional perspective

Anna Jansen, European Paediatric Neurology Society (EPNS)

Michelangelo Mancuso, European Academy of Neurology (EAN)

Regulatory perspective

Belgian EU presidency perspective

12.35-12.40 Wrap up and closing remarks

Frédéric Destrebecq, European Brain Council, Executive Director

12.40-14.00 Networking Lunch

