Towards a Rare Brain Disease Ecosystem

Day: 29th February 2024 – Rare Disease Day

Duration: 09.30 - 14.00

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

Aim

The main purpose of Rare Disease Day is to raise awareness about rare diseases and their impact on the lives of those affected. In the continuity of the European Brain Council's (EBC) activities on rare brain diseases, EBC introduced the concept of a Rare Brain Disease Ecosystem to engage key stakeholders in the field to identify gaps, priorities, and projects in Europe. During this event, researchers, clinicians, persons living with brain disease and industry will share their perspectives and priorities in the rare brain disease research, care, and policy space, and will brainstorm about highly needed projects to be implemented.

Agenda

9.30 - 10.00	**Registration & Welcome Coffee**
Session 1:	**Welcome and Opening: The Need for a Rare Brain Disease Ecosystem**
10.00 - 10.05	**Welcome**
	Frédéric Destrebecq, European Brain Council
10.05 - 10.10	**Opening Address (video message)**
	MEP Stelios Kympouropoulos (GR, EPP)
10:10 - 10:25	**The MOONSHOT initiative**
	Magda Chlebus, European Federation of Pharmaceutical Industries and Associations (EFPIA)
Session 2:	**Patient and caregiver priorities**
10.25 - 10.35	**A personal 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	**Rare Neuroimmune Diseases**
	Stephanie Ludwig, Roche (TBC)
11.15 - 11.25	**Amyotrophic Lateral Sclerosis, a rare neurodegenerative disease**
	Juan Jose Fernandez Romero, Amylyx Pharmaceuticals
11.25 - 11.40	**Coffee Break**
Session 4:	**Clinical care pathways for rare disease in Europe: Panel Discussion**
11.40 - 12.20	*Moderator: Vinciane Quoidbach, European Brain Council*
	Paola Giunti, Ataxia UK: The case of ataxia
	Anita MacDonald, Birmingham Children's Hospital (UK): The case of phenylketonuria
	Giuseppe Turchetti, Scuola Superiore Sant'Anna (IT): Towards a digital care pathway?
Session 5:	**Unmet needs: Open Discussion**
12.20 - 12.50	*Moderator: Frédéric Destrebecq, European Brain Council*
	Anna Jansen, European Paediatric Neurology Society (EPNS)
	Michelangelo Mancuso, European Academy of Neurology (EAN)
	Interaction with the Audience: Q&A
12.50 - 13.00	**Wrap up and closing remarks**
	Frédéric Destrebecq, European Brain Council
13.00 - 14.00	**Networking Lunch**