

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)	<i>Matthias Heck, Alexion Pharmaceuticals</i>
11.05-11.15	Rare Neuroimmune Diseases	<i>TBC</i>
11.15-11.25	Amyotrophic Lateral Sclerosis, a rare neurodegenerative disease	<i>Lugdivine Le Dez, Amylyx Pharmaceuticals</i>
Session 4:		Clinical care pathways for rare disease in Europe
11.25-11.35	The case of ataxia	<i>Paola Giunti, Ataxia UK</i>
11.35-11.45	The case of phenylketonuria	<i>Anita MacDonald, Birmingham Children's Hospital, UK</i>
11.45-11.55	Towards a digital care pathway?	<i>Giuseppe Turchetti, Scuola Superiore Sant'Anna, IT</i>
11.55-12:05	Bringing Artificial Intelligence home for a better care of amyotrophic lateral sclerosis	<i>TBC</i>
Session 5:		Open Discussion: Unmet needs
12.05-12.35	Healthcare professional perspective	<i>Anna Jansen, European Paediatric Neurology Society (EPNS)</i> <i>Michelangelo Mancuso, European Academy of Neurology (EAN)</i>
	Regulatory perspective	
	Belgian EU presidency perspective	
12.35-12.40	Wrap up and closing remarks	<i>Frédéric Destrebecq, European Brain Council, Executive Director</i>
12.40-14.00	Networking Lunch	



Meet the speakers



**Frédéric Destrebecq, Executive Director,
European Brain Council**

Frédéric Destrebecq is the Executive Director of the European Brain Council since October 2014. In this capacity, he is responsible for providing strategic direction and leadership while managing the day to day operations of EBC and its ongoing relationships with its member associations and other stakeholders, as well as representing the organisation in various European and national forums.

Fred holds a Master Degree in Political Science and International Relations from the Université Catholique de Louvain (Belgium). He also studied at the Institut d'Etudes Politiques (Paris) and University of Wales College (Cardiff), in the framework of the former EU Socrates exchange programme. Prior to EBC, Fred served the European Union of Medical Specialists (UEMS) as Chief Executive Officer, and previously as Director for European Affairs.



Tim Buckinx, Founder & CEO, Epihunter

With more than a decade steering global digital initiatives at Bose, Tim Buckinx is no stranger to the power of technology. But it's his personal journey as a father to a son living with refractory epilepsy that truly ignited his passion. Driven by this deep personal connection, Tim founded epihunter, a trailblazing digital therapeutics company aimed at transforming lives.

His vision? To leverage the untapped potential of digital technology in diminishing the everyday challenges posed by neurological disorders. Imagine glasses, which don't cure bad vision but drastically reduce its impact, enabling people to engage fully with the world. Now, what if digital solutions could do the same magic for those grappling with brain disorders? Tim Buckinx believes they can—and he's on a mission to prove it.





Orla Gavin, Executive Director, European Federation of Neurological Associations

Dr. Orla Galvin came to patient advocacy with a PhD in Medicine and background in drug discovery and design in both academic and industry environments. Transitioning to patient advocacy work as Director of Research and Policy, Orla led high impact, multi-stakeholder socio-economic studies, patient preference studies, and accessibility studies across the globe assessing both rare and common conditions.

Orla is an internationally invited speaker to EU Parliament, WHO-forums, research and clinical learned societies, patient organisations, and industries on topics such as:

- Patient and public involvement in advocacy, research and policy,
- Research in advocacy and policy/evidence-based advocacy,
- Education in advocacy,
- Generation of real-world data,
- Patient reported outcomes, and
- Health economics.



Giuseppe Turchetti, Co-Founder of the Institute of Management of the Scuola Superiore Sant'Anna (SSSA - Pisa)

Prof. Giuseppe Turchetti received his Laurea Degree in Economics from the University of Pisa. He received his PhD in Management from the Scuola Superiore Sant'Anna in Pisa, where he is professor of Economics and Management of Innovation and Healthcare. Fulbright Scholar, he spent several years for research in USA as Visiting Scholar at The Wharton School of the University of Pennsylvania (Philadelphia), at the Illinois Institute of Technology (Chicago) and at the Kellogg School of Management of the Northwestern University (Chicago). He is Co-Founder of the Institute of Management of the Scuola Superiore Sant'Anna (SSSA - Pisa), of the Research Center on Technologies and Services for the Support of Longevity (SSSA - Pisa), of the Research Center on European Transplantation Management (SSSA - Pisa), of the Center of Excellence ENDOCAS (on Computer Assisted Surgery) of the University of Pisa. His main research interests are in the fields of the: organisation, financing and evaluation of healthcare services and health technologies, of the management of innovation, organization and commercialization of medical technologies (pharma, medical devices, e-health), strategic management and marketing in the healthcare sector. His research addresses many therapeutic areas and the wide field of rare diseases. He is working on/coordinating several national and international projects in the area of healthcare technologies and management. He collaborates in several projects within the ERN Program (on a regular basis with ERN ReCONNET), European Commission. He is author/editor of twenty books and of three hundred scholarly papers and book chapters..





Michelangelo Mancuso, Co-chair of the EAN Coordinating Panel on Rare Neurological Diseases, European Academy of Neurology

Professor Mancuso is the head of the Centre of Neurogenetics and expertise for mitochondrial diseases and rare diseases at the Neurological Clinic of the University Hospital of Pisa (Orphanet Center EUGTIT247621).

Scientific and research activity of Professor Michelangelo Mancuso has mainly been conducted in the field of mitochondrial, neurogenetics and stroke. As a whole result, he published more than 300 full papers on peer-reviewed Life Science/Current Contents cited scientific journals. According to Scopus, Dr Mancuso's articles have more than 18000 citations, and the H-Index is 54.

Mancuso is the co-chair of the EAN Coordinating Panel on Rare Neurological Diseases, Past-Coordinator of the Neurogenetics Group of the Italian Society of Neurology and past-chair of the Neurogenetics Panel of the European Academy of Neurology. Recently, Prof Mancuso has been nominated co-chair of the mitochondrial working group of the ERN NMD, and chair of the inter-ERN mitochondrial working group.

