



Wednesday, 23 March 2022
14:00 – 15:00 (CET)

Webinar:

**RARE PAEDIATRIC NEUROLOGICAL DISEASES:
 A FOCUS ON EUROPE**

PTC Therapeutics invites you to participate in a multi-stakeholder webinar to present the findings of the Economist Impact* report ‘Rare paediatric neurological diseases: A focus on Europe’ and discuss how policymakers, healthcare providers and patients can collaborate to improve care and outcomes.

Rare paediatric neurological conditions are debilitating diseases which significantly impact the day-to-day lives of children and their families. Most rare diseases have neurological symptoms, and about 90% of those affecting children have major neurological effects limiting the ability to achieve key cognitive and physical development milestones.

To highlight policy priorities for improving care, patient outcomes and quality of life, Economist Impact has undertaken a project to better understand how these disorders affect the lives of patients and their families. The resulting report ‘**Rare paediatric neurological diseases:**

A focus on Europe’ highlights the difficulties in achieving a timely and accurate diagnosis, as well as the numerous challenges to providing adequate care and support. It concludes that policymakers should work to develop a rare diseases healthcare ecosystem that is sufficiently resourced and organised to enable even the most complex conditions to be assessed and managed expeditiously.

MODERATED BY:



Elizabeth Sukkar

Senior Research Manager in Global Health, Economist Impact.

SPEAKERS:



Prof. Dr. Tim Cox

Professor of Medicine, Emeritus and Director of Research, University of Cambridge; Lead Patron, MLD (Metachromatic Leukodystrophy) Support Association UK



Prof. Dr. Maurizio Scarpa

Coordinator, European Reference Network for Hereditary Metabolic Diseases (MetabERN), Director of the Regional Coordinating Center for Rare Diseases Udine University Hospital, Udine, Italy



Tobias Mentzel

Chairman, European Leukodystrophy Germany (ELA Deutschland e.V.).



Dorica Dan

Chair, Romanian Prader Willi Association; President, the Romanian National Alliance for Rare Diseases; Member, EURORDIS Board of Officers and Board of Directors



Ivana Badnjarević

President, Lil’ Brave One Association (Hrabriša), Serbia



Thomas Bols

Head of Government Affairs and Patient Engagement, EMEA & APAC, PTC Therapeutics

Organised by PTC Therapeutics

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* Economist Impact (EI) is part of The Economist Group. Economist Impact combines the rigour of a think-tank with the creativity of a media brand to engage a globally influential audience. EI believes that evidence-based insights can open debate, broaden perspectives and catalyse progress.

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