Patient Reported Outcomes for Multiple Sclerosis

The impact of PROMs in developing emerging therapies

12 November 2025 (8:30–13:00 CET)

AIM: Promote collaboration across stakeholders to develop a consensus-driven foundation to integrate Patient Reported Outcomes Measures (PROMs) into phenotype classification of people with MS with ultimate goal of accelerating therapeutic development.

Objectives:
- Review current EMA regulatory models regarding the use of Patient Experience Data (PED) including PROMs in drug approval;
- Develop Consensus on PROM Integration into phenotype classification of people with MS and new clinical descriptors of MS;
- Create a roadmap for future action for MS and beyond.

Location: Grand Hotel Dino - Baveno, Italy (side event) during the 33rd Annual Meeting of the European Charcot Foundation (ECF)
Duration: 4 hours, 08:30 – 13:00 CET
Registration link: https://www.etr.events/PROMS

Scientific Planning Committee co-chairs:
- **Daniel Ontaneda**, Cleveland Clinic Mellen Center for Multiple Sclerosis
- **Maria Pia Amato**, PROMS WG 1 Co-Leader

Scientific Planning Committee members:
- **Elena H. M. de Lapiscina**, European Medicine Agency
- **Kathleen Zackowski**, National MS Society
- **Patrick Vermersch**, PROMS SSC Co-Chair
- **Paola Zaratin**, PROMS SSC Co-Chair
- **Anne Helme**, PROMS ECT Co-Chair
- **Jeremy Hobart**, PROMS WG2 Co-Leader
- **Carmen Tur**, PROMS WG3 member
- **Paolo Cortesi**, PROMS WG4 Co-leader

Agenda

Welcome Session and Introduction

Time	Duration	Session Title	Speakers	
8:30-8:45	15′	**Welcome**	Hans-Peter Hartung	Heinrich-Heine University Düsseldorf
			Xavier Montalban	Centre d'Esclerosi Múltiple de Catalunya (Cemcat)
			Lydia Makaroff	MS International Federation
		Lived experience perspective The urgent need for relevant PROMs to improve therapy development and access	Helga Weiland PROMS ECT Co-Chair	
8:45-8:55	10′	**Meeting agenda and aims** Overview of meeting objectives	Paola Zaratin Patrick Vermersch	Italian MS Society Université de Lille

Context: The path forward to increase the uptake of PROMs, "meaningfulness to the patient's health", in the context of new clinical descriptors without undermining the rigor of EMA PROMs validation and use in decision-making.

Session 1: **EMA Reflection Paper on Patient Experience Data in Q2-3 2025**

Moderators: Elena H. Martínez-Lapiscina European Medicines Agency Mario Alberto Battaglia Italian MS Society

8:55-9:15	20′	Keynote Speech: **The Value of Patient Experience Data (PED) in Medicines Development and Regulation: An EU Perspective**	Juan Garcia Burgos Stakeholders and Communication Division, European Medicines Agency (EMA)
9:15-9:25	10′	**ECT response to the EMA reflection paper**	Meenakshi Nandakumar PROMS ECT
9:25-9:45	20′	**Discussion** • Implications of reflection paper for PROMs in MS; • When and how to engage EMA (e.g., Innovation Task Force); • Differences in evidence standards for various regulatory goals.	

Session 2: **Clinical descriptors and new classification - Bridging biological disease mechanisms with different clinical states: the role of PROMs in phenotyping MS**

Moderators: Patrick Vermersch Université de Lille Maria Pia Amato Università di Firenze

9:45-9:55	10′	**Lived experience perspective on MS classification**	Marilena Hadjiyianni PROMS ECT
9:55-10:10	15′	**Redefining MS disease descriptors and phenotypes**	Alan Thompson University College London
10:10-10:25	15′	**New clinical descriptors rooted on PROMs: myth or reality**	Giampaolo Brichetto Italian MS Society
10:25-10:40	15′	**Redefining Rating Scale Data Analysis in MS Trials: Report from the PROMS Workshop**	Jeremy Charles Hobart University of Plymouth
10:40-11:00	20′	**Discussion** • Role of PROMs in capturing progression beyond relapses; • The case for phase-specific and phenotype-specific PROMs; • Aligning biological and experiential data; • PROMs standardisation has to happen together with their personalized use.	
11:00-11:20	20′	**Coffee break**	

Session 3: **Alignment of stakeholders for PROs for emerging therapies**

Moderators: Xavier Montalban Centre d'Esclerosi Múltiple de Catalunya (Cemcat) Nektaria Alexandri Merck

11:20-11:35	15′	**Successful PROs development from rare neurological diseases: the case of Myasthenia Gravis (MG)**	Carlo Antozzi Istituto Neurologico Carlo Besta
11:35-11:50	15′	**Industry perspective on integration of PROs to drug development**	Tammy McIver Roche
11:50-12:05	15′	**Regulatory perspective on alignment of PROs and drug development**	Elena H. Martínez-Lapiscina European Medicines Agency
12:05-12:15	10′	**Discussion** • Lessons from Brain Health (Nektaria Alexandri) • Lessons from Myasthenia Gravis: Strategies to adapt existing approaches to MS-specific contexts • Limitations of Current Tools (EDSS) & Impact on Industry: PROMs as primary endpoints – myth or reality? • Key regulatory hurdles and opportunities for PROMs use in MS	

Session 4: **Potential roadmap and next steps**

Moderator: Lydia Makaroff MS International Federation

12:15-12:25	10′	**Lived experience perspective: call to action**	R van den Bos PROMS ECT
12:25-12:55	30′	**Discussion panel**	Panel Members:

Moderator:
Daniel Ontaneda
Cleveland Clinic

Timothy Coetzee	United States MS Society
Anne Helme	PROMS ECT Co-Chair
Jeremy C. Hobart	University of Plymouth
Agnė Kazlauskaitė	Roche
Elena H. Martínez-Lapiscina	European Medicines Agency (EMA)
Alan Thompson	University College London
Carmen Tur	Cemcat
Patrick Vermersch	Université de Lille
Paola Zaratin	Italian MS Society

Session 5: **Closure**

12:55-13:00	5′	**Closing remarks**	Hans-Peter Hartung Heinrich-Heine University Düsseldorf