

Understanding the Patient Experience: Cognitive Function, QoL, and Narrative-Based Outcomes in Mucopolysaccharidosis

Thursday, September 4, 2025 | 1:45–2:45 PM
Sponsored Seminar 4 | Room 6 (Sakura Room)
Kyoto International Conference Center



We look forward to seeing you at the JCR booth (#9) and Seminar 4

WELCOME AND INTRODUCTION



CHAIRPERSON

Roberto Giugliani, MD, PhD
Federal University of Rio Grande do Sul
Porto Alegre, Rio Grande do Sul, Brazil



CHAIRPERSON

Takashi Hamazaki, MD, PhD
Osaka Metropolitan University Graduate School of Medicine
Osaka, Japan

OVERVIEW OF MUCOPOLYSACCHARIDOSIS (MPS) DISEASE STATE



Nicole Muschol, MD
University Medical Center Hamburg-Eppendorf
Hamburg, Germany

LIVING WITH PATIENTS AND OBSERVING SUBTLE CHANGES IN QoL MEASURES



Terri Klein, CNPM, MPA
National MPS Society
Durham, North Carolina, United States

PATIENT-REPORTED OUTCOMES IN MPS



Ana Maria Martins, MD, MSc, PhD
Federal University of São Paulo
São Paulo, Brazil

PANEL DISCUSSION

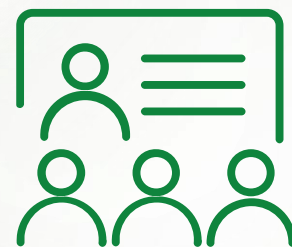
All (with questions submitted from the audience)

Mucopolysaccharidoses (MPS) are a group of inherited metabolic rare diseases caused by the deficiency of enzymes required to break down glycosaminoglycans (GAGs) in the lysosome. GAG accumulation can lead to damage to multiple organs and, in severe cases, progressive cognitive decline. In this symposium, we will review the current state of MPS and explore in depth how changes in quality-of-life measures and other patient-reported outcomes can influence research and patient care.



QoL, quality of life

This satellite symposium is fully funded and organized by ICIEM2025 and JCR Pharmaceuticals Co., Ltd., and is intended for health care professionals only. JCR Pharmaceuticals medicines may be discussed at this meeting. Some content may be deemed promotional in your territory.



Blood-brain barrier penetrating AAV vector for the treatment of CNS diseases

Date
and
Time

**September 5, 2025
12:00-13:00**

Venue

Room 3 (Room B-1)

Kyoto International Conference Center
(422 Iwakura Osagicho, Sakyo-ku, Kyoto, 606-0001, Japan)

Chair

Maurizio Scarpa, MD, PhD

Director, Regional Coordinating Center for Rare Diseases
Udine University Hospital, Udine, Italy

Coordinator, European Reference Network For Hereditary
Metabolic Diseases (MetabERN)

Professor of Pediatrics University of Padova Italy

Speaker

Hiroyuki Sonoda, PhD

Senior Vice President, Head of Research
JCR Pharmaceuticals Co., Ltd.