

EVENT FOR PROFESSIONALS:

Role of biomarkers in advancing access to treatments for individuals with neurocognitive or neuronopathic MPS conditions

Tuesday, 18 March 2025, 9.30am - 4.30pm (CET)
Renaissance Hotel, Schiphol Airport, Amsterdam and online

Developing treatments for individuals with progressive neurocognitive or neuronopathic diseases such as Mucopolysaccharidosis (MPS) presents several significant challenges.

Biomarkers can, however, play a critical role in assessing the efficacy of treatments, by providing objective measures of disease progression or response to therapy.

The aim of this meeting is to bring clinical and scientific experts, together with regulators, decision makers, commissioners, industry and patient organisations to facilitate the global sharing of information and discussion and to highlight the importance of integrating biomarkers into regulatory frameworks.



Hosted by:

MPS Society (UK) in association with International MPS Network

Chairs:

Prof Simon Jones
Prof Maurizio Scarpa

Invitation for professionals:

Regulators, country specific decision makers, medics, industry, patient group representatives, HTA bodies and commissioners



Proposed agenda*

9.00 Welcome and introduction

Part 1: Clinical & Patient perspective

9.20 Clinical description of MPS

9.30 What about the neurology?

9.40 The uncertain path towards approval

10.10 Clinical trials need biomarkers

10.20 Discussion

10.30 *Break*

Part 2: What does the science tell us?

(Rationale for Heparan sulfate as a biomarker)

10.50 Development of heparan sulfate assays

11.00 Gathering evidence to support heparan sulfate as a potential surrogate marker

11.10 The role of heparan sulphate in advancing clinical trial development

11.20 Impact of trial failure on clinicians and patients

11.30 Discussion

11.45 *Lunch*

Part 3: Currently accepted and evolving evidence

12.45 Clinical trial updates

13.45 Discussion

Part 4: Views from the Regulators

14.05 Global regulatory view

14.20 Reagan-Udall Foundation presentation

14.35 FDA presentation

14.50 *Break*

15.10 EMA presentation

15.25 MHRA presentation

15.40 Discussion

Part 5: Panel Discussion & Recommendations

16.00 Panel discussion & recommendations

16.20 Closing remarks

16.30 *End*

Scan me



mpssociety.org.uk/role-of-biomarkers

Attend in
person or
online

**subject to change*