Workshop on Clinical Outcome Measures and endpoints for efficacy assessment in spinal muscular atrophy

October 13th at European Medicines Agency, London, UK
Room 2A

8:30 Arrival and Registration

9:00 Welcome and Introduction
Eric Abadie (CHMP Chair) and Spiros Vamvakas (Acting Deputy Head of Sector for Scientific Advice and Orphan Drugs)
Volker Straub (Newcastle University, TREAT-NMD Coordinator)
Cynthia Joyce (SMA Foundation/ ICC)

9:35 Patient Perspective
Peter Streng (ENMC, TREAT-NMD)

9:45 Agency expectations on outcome measures
Rembert Elbers (BfArM, member of the COMP and SAWP)

10:05 What is Spinal Muscular Atrophy
Enrico Bertini (FTELE, TREAT-NMD)

10:25 Future Perspectives for clinical Trials in SMA
Francesco Muntoni (Institute of Child Health, UCL, TREAT-NMD)

10:45 Coffee

Session I – Spinal Muscular Atrophy Type I
Chairs: Eric Abadie (CHMP Chair)

11:00 Clinical Presentation SMA Type I
Janbernd Kirschner (Clinical Trial Coordination Centre, TREAT-NMD)

11:15 Scientific consensus on outcome measures for SMA Type I
Richard Finkel (Director, Neuromuscular Program, The Children's Hospital of Philadelphia)

11:30 Discussion and consolidation

Session II – Non-ambulant patients with Spinal Muscular Atrophy
Chair: Daniel Brasseur (PDCO Chair)

12:00 Clinical Presentation of non-ambulant SMA
Petra Kaufmann (Associate Director, Pediatric MDA Neuromuscular Clinic, Columbia University)

12:15 Scientific consensus on outcome measures for non-ambulant SMA
Eugenio Mercuri (FTELE, TREAT-NMD)

12:30 Discussion and consolidation

13:00 Lunch break

Session III – Ambulant Patients with Spinal Muscular Atrophy
Chair: Kerstin Westermark (COMP Chair)

14:00 Clinical Presentation SMA Ambulatory Type
Klaus Zerres (Department of Human Genetics, Aachen University of Technology)

14:15 Scientific consensus on outcome measures for SMA Type III
Julaine Florence(Director of Clinical Studies in Neuromuscular Diseases, Washington University School of Medicine)

14:30 Discussion and consolidation

Session IV – Biomarkers in Spinal Muscular Atrophy
Chair: Member of FDA

15:00 Overview of biomarkers in SMA
Louise Simard (Head of Biochemistry and Medical Genetics, University of Manitoba)

15:15 Scientific consensus on Biomarkers for SMA
Christina Brahe (Istituto di Genetica Medica, Università Cattolica S Cuore)

15:30 Electrophysiology as a biological marker
Kathy Swoboda (Head of the Pediatric Motor Disorders Research Program, University of Utah School of Medicine)

15:45 Discussion and consolidation

16:00 Coffee break

Session V – Readiness for clinical Trials in SMA
Chairs: AFM representative and Kate Bushby (TREAT-NMD Coordinator)

16:20 Summary and Discussion
Kate Bushby (Newcastle University, TREAT-NMD Coordinator),
Stefanie Possekel (Santhera, TREAT-NMD Activity Leader),
Domenico Marchetti (SMA Europe),
Rembert Elbers (BfArM, member of the COMP and SAWP)

17:20 Concluding Remarks
EMEA and TREAT-NMD representatives

17:30 End of meeting

The elements originating during the discussion will not constitute a formal specific advice on a particular product or class of products. The positions expressed by the Experts and the EMEA during this workshop will not be regarded as binding in relationship to any aspect of subsequent institutional work before to be endorsed by the CHMP and/or the PDCO.

Additional Information

Websites

EMEA http://www.emea.europa.eu/
TREAT-NMD http://www.treat-nmd.eu

If you should have any questions or queries relating to the organisation of the meeting please contact the TREAT-NMD Coordination Office at emma.heslop@ncl.ac.uk or silja.sommer@emea.europa.eu

If you have any queries regarding the scientific content of the meeting please contact domenicantonio.tropepi@emea.europa.eu or janbernd.kirschner@uniklinik-freiburg.de