



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

Volker Straub (Newcastle University, TREAT-NMD Coordinator)

Cynthia Joyce (SMA Foundation/ ICC)

Eric Abadie (CHMP) and Spiros Vamvakas (Acting Head of Sector for SAOD)

9:30 Patient Perspective

Peter Streng (ENMC, TREAT-NMD)

9:40 Agency expectations on outcome measures

Rembert Elbers (BfArM, member of the COMP and SAWP)

10:00 What is Spinal Muscular Atrophy

Enrico Bertini (FTELE, TREAT-NMD)

10:20 Future Perspectives for clinical Trials in SMA

Francesco Muntoni (Institute of Child Health, UCL, TREAT-NMD)

10:40 Coffee

Session I – Spinal Muscular Atrophy Type I

Chairs: Eric Abadie (CHMP)

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 Bresseur (PDCO)

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)

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 Possek (Santhera, TREAT-NMD Activity Leader),
Domenico Marchetti (SMA Europe),
Rembert Elbers (BfArM, member of the COMP and SAWP)*

17:20 Concluding Remarks

EMA and TREAT-NMD representatives

17:30 End of meeting

The organizers would like to thank the following organizations for their financial support of this workshop and the ICC for its organizational support.



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>

Directions to Meeting

Address:

7 Westferry Circus

Canary Wharf

London E14 4HB

Tel. (44-20) 74 18 84 00

Fax (44-20) 74 18 84 16

Map / Instructions:

http://www.emea.europa.eu/pdfs/general/direct/EMEA_location_Map.pdf

To plan your journey using the London Underground, please visit the website:

www.tfl.gov.uk/tube/

Journey Planner 1 2 3

Route details English Go

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Journey Summary

Departing: Thursday 03 July 2008 at: 08:00
 From: Kings Cross Station
 To: E14 4HB
 Restrictions:

Time	Details	Information
07:58	King's Cross St.Pancras Underground Station	Average journey time: 12 mins
08:10	Take the Northern Line towards Morden	Zone(s): 1
	LONDON BRIDGE STATION: Reduced escalator service until mid October.	
	Zone(s): 1	
08:16	London Bridge Underground Station	Average journey time: 8 mins
08:24	Take the Jubilee Line towards West Ham or Jubilee Line towards Stratford or Jubilee Line towards North Greenwich	Zone(s): 1, 2
	CANARY WHARF STATION: Reduced escalator service until late August.	
	Zone(s): 1, 2	
	Canary Wharf	Transfer time: 14 mins
	Walk to E14 4HB	
08:38	E14 4HB	

Maximum journey time: 00:38
 Interchanges: 1
 Not what you expected? [Click here for information on planned service disruptions.](#)
 Please check ticket options and fare prices at <http://www.tfl.gov.uk/faresandtickets>.