



4 November 2016
EMA/726250/2016
Human Medicines Evaluation Division

Spinal muscular atrophy stakeholder workshop - programme

Organized by SMA Europe, TREAT-NMD and EMA

11 November 2016 at the European Medicines Agency, 30 Churchill Place, Canary Wharf, London, UK, Meeting room 2A

	Programme	Presenter
9:00	Registration and Coffee	
9:30	Welcome	Serge Braun and Jordi Llinares
Session 1 - Setting the Scene		Chairs: Thomas Voit and Karl Broich
9:40	Parent perspective: impact of SMA	Mencia De Lemus
9:55	SMA prevalence, diagnosis, classification and genetic mechanisms	Brunhilde Wirth
10:10	General overview of novel experimental therapeutic approaches	Francesco Muntoni
Session 2 - Type 1 SMA		Chairs: Jan Kirschner and Pavel Balabanov
10:25	Patient perspective to clinical benefit in type 1 SMA	Elliot Duffy
10:35	Natural history/impact of standards of care on survival and function	Richard Finkel
10:50	Outcome measures	Basil Darras
11:05	Biomarkers	Arthur Burghes
11:20	Break	



Programme		Presenter
11:40	Regulatory perspective	Marion Haberkamp
12:00	Industry perspective	Kathie Bishop
12:15	Discussion and questions	Richard Finkel and Eduardo Tizzano
12:45	Lunch	
	Session 3 - Ambulant & non ambulant type 2 & 3 SMA	Chairs: Natalie Goemans and Maria Isaac
13:45	Registries, and trial readiness	Hanns Lochmuller
14:00	Natural history and trajectories	Eugenio Mercuri
14:15	Standards of care	Richard Finkel
14:35	Overview of Outcome measures	Anna Mayhew and Jacqueline Montes
14:55	Exploratory outcome measures (including MRI)	Laurent Servais
15:05	Patient perspective to clinical benefit	Kenneth Hobby, Luca Binetti and Elena Mazzone
15:25	Break	
15:45	Regulatory perspective	Mario Miguel Rosa
16:00	Industry perspective	Omar Kwaja
16:15	Discussion	Richard Finkel
16:50	Concluding remarks	Eugenio Mercuri & Manuel Haas
17:00	End of meeting	