

Friday 10 May 2019

12.30 – 14.00	Lunch
14:00 – 14:45	Chair: L. Servais/E. Tizzano
14.00 – 14.15	Welcome by Dr A. Breukel
14:15 – 14.30	Introduction and aim of the meeting, L. Servais, E. Tizzano
14:30 – 14:45	Introduction participants, All
14:45 – 16:15	Session 1 Chair: L. Servais
14:45 - 15:00	genetic modifier/prediction, E. Tizzano
15:00 – 15:15	Inhouse techniques for screening test: validation, F. Boemer
15:15 - 15:45	SMA Cost + the view of economist on screening, M. Hilligsman
15:45 - 16:15	The view of psychologists and ethic on screening, P. Young
16:15 - 16:30	BREAK
16:30 – 18:00	Session 2, Chairs E. Bertini, L. Servais, E. Tizzano
16:30 - 16:45	Nurture data, E. Bertini
16:45 - 17:00	Roche pre-symptomatic program, K. Gorni
17:00 - 17:15	Avexis pre-symptomatic program, I. Kausar
17:15 - 18:00	Discussion and Wrap up, all
19:00	Dinner

Saturday 11 May 2019

09:00 – 14.45

Session 3, Chair D. Tiziano

09:00 - 09:15

NBS in NY and USA, K. Stephenson

09:15 - 09:30

NBS in Belgium L. Servais

09:30 - 09:45

NBS in Germany, W. Mueller-Felber

09:45 – 10:00

NBS in Italy, D. Tiziano

10:00 - 10:15

NBS in Cataluna, E. Tizzano

10:15 - 10:45

Overview and comparison of NBS, L. Servais

10:45 - 11:00

BREAK

11:00 - 12:30

Session 4, Chair, W. Mueller-Felber

11:00 – 11:15

Genetic neonatal screening, A. Burghes

11:15 – 11.30

MDA experience: other diseases, K. Stephenson

11:30 – 11.45

Association with other screening tests, F. Boemer

11.45 – 12:00

Extension towards other EU countries, K. Rucinski

12:00 – 12:15

Perkin Almer screening test, M. Hjort

12:15 – 12:30

Roche screening test, K. Gorni

12:30 – 13:00

Discussion, L. Servais/E. Tizzano

13:00 - 14:00

Lunch

14:00 – 15:50

Session 5 Chair L. Servais/E. Tizzano (no pharmaceutical representatives)

14:00 - 14:20

Alternative to NBS / carrier screening? tbn

14:20 - 14:40

Treatments and number of copies, L. Servais

14:40 - 15:00

Discussion, all

Program Saturday 11 May, continued

15:00 - 15:10	Patients advocacy group Spain, M. de Lemus
15:10 - 15:20	Patients advocacy group US, K. Stephenson
15:20 - 15:30	Patients advocacy group Russia, O. Germanenko
15:30 – 15:40	Patients advocacy group Poland, K. Rucinski.
15:40 – 15:50	Patients advocacy group France, L. Ouillade
15:50 - 16:10	BREAK
16.10– 18:00	Session 6, Chair L. Servais, E. Tizzano (no pharmaceutical representatives)
16:10 - 18:00	Follow up of patients screened, all
19:00	Dinner

Sunday 12 May 2019

09:00 – 10:30	Session 7, Chairs L. Servais, E. Tizzano (no pharmaceutical representatives)
09:00 09:15	Strategy for dissemination, T. Dangouloff
09:15 – 10:30	Patients long term follow up (registry...) (1), All
10:30 - 10:45	BREAK
10:45 – 12:00	Session 8, continued, Chairs L. Servais, E. Tizzano
10.45 – 11.45	Patients long term follow up (registry...) (next), All
11:45 - 12:00	Conclusion and wrap up, L. Servais, E. Tizzano
12:00 – 13:00	Lunch and departure