

Programme

Friday 11 October 2019

12.30 – 14.00	<i>Lunch</i>
14.00 – 14.30	Welcome
14.00 – 14.30	Introduction (ENMC and co-organizers)
14.30 – 16.00	Session 1: Summary and Overview of former clinical DM workshops, Chair Benedikt Schoser
14.30 – 14.50	Summary of former ENMC DM workshops, Benedikt Schoser
14.50 – 15.10	Summary of former Marigold DM brain workshops, Giovanni Meola
15.10 – 15.30	Summary of CDM aspects, Anne-Berit Ekström
15.30 – 16.00	Discussion, all
16.00 – 16.30	<i>Break</i>
16.30 – 17.00	Session 2: First clinical trial in DM, Chair Baziel van Engelen
16.30 – 17:00	Summary of results of The OPTIMISTIC trial, Baziel van Engelen
17.00 – 18.30	Session 3: Recent advances in genetics in DM, Chair Rick Wansink
17.00 – 17.30	New aspects of the DM1 genotype, Darren Monckton
17.30 – 18.00	New aspects of the DM2 genotype, Annalisa Botta
18.00 – 18.30	RNA products from the DM loci, Rick Wansink
19.00	<i>Dinner</i>

Saturday 12 October 2019

09.00 – 10.30	Session 4: Basic science of the DM repeat mutations Chair Genevieve Gourdon
09.00 – 09.30	RNA mechanisms in DM, Krzysztof Sobczak
09.30 – 10.00	New aspects of mechanisms in CDM, Geneviève Gourdon
10.00 – 10.30	Discussion
<i>10.30 – 11.00</i>	<i>Break</i>
11.00 – 13.00	Session 5: Disease models in DM, Chair Genevieve Gourdon
11.00 – 11.30	Immortalized myoblast cell culture, Denis Furling
11.30 – 12.00	DM myoblast and myotube characterisation, Peter Meinke
12.00 – 12.30	miRNA-based therapeutics for myotonic dystrophy, Ruben Artero
12.30 – 13.00	DM mouse models, Mario Gomes-Pareira
<i>13.00 – 14.00</i>	<i>Lunch</i>
14.00 – 18.30	Session 6: Therapeutic approaches in cell and animal models for DMs, Chair: Rick Wansink
14.00 – 14.30	DM1 in human pluripotent stem cells, Karen Sermon
14.30 – 15.00	iPSCs, Cecile Martinat
15.00 – 15.30	Cell-based therapy, Hans van Bokhoven
15.30 – 16.00	CRISPR/Cas, Thierry VandenDriessche
<i>16.00 – 16.30</i>	<i>Break</i>
16.30 – 17.00	Antisense oligonucleotides, David Brook
17.00 – 17.30	Small molecules, Michael Sinnreich
17.30 – 18.00	Splice regulators beyond Muscleblind, Nicolas Charlet-Berguerand
18.00 – 18.30	Muscleblind and miRNAs, Jonathan Hall
<i>19.30</i>	<i>Dinner</i>

Sunday 13 October 2019

09.00 – 12.30	Session 6: Framing a European DM research network, Chairs: Baziël van Engelen, Benedikt Schoser
09.00 – 09.15	General structure, organization and challenges of a network, Rick Wansink/ Benedikt Schoser
09.15 – 09.30	Coordinated basic science research for molecular trials, Rick Wansink/Genevieve Gourdon
09.30 – 09.45	Discussion
09.45 – 10.00	Coordinated clinical research trials for DMs: unmet needs, Baziël van Engelen/ Benedikt Schoser
10.00 – 10.15	Congenital/juvenile DM: unmet needs, Anne-Berit Ekström
10.15 – 10.30	Discussion, all
<i>10.30 – 11.00</i>	<i>Break</i>
11.00 – 11.30	DMs - the patient and family perspective, Alain Geille/ Bas Haasakker/ Nathalie Loux
11.30 – 11.45	Preparation of lay summary, Benedikt Schoser/ Rick Wansink
11.45 – 12.30	Group discussion: European DM research network (defining a worksheet and plan of action)
<i>13.00</i>	<i>lunch</i>