5th of February

09:00 - 10:30	*Registration – Main hall*
10:30 - 11:00	**Welcome** - Auditorium Mencía de Lemus - SMA Europe President & Professor Kevin Talbot, SMA Europe SAB vice-Chair
11:00 - 12:00	**Together to treat SMA**- Auditorium "SMArt Expectations - Results from the European Patient Expectation Survey 2019 (EUPESMA-2019)" Dr. Nicole Gusset, vice - President of SMA Europe "From gene discovery to treatments" Professor Judith Melki
12:00 - 13:30	*Lunch Break -Main hall & Crick room (1st floor)*
12:15 - 13:15	*Symposium: Roche: Perspectives – the impact and understanding that matters- Auditorium*
13:30 - 15:15	**Session 1 - Emerging treatments & novel approaches in therapy, Chaired by Dr. Mandana Arbab**- Auditorium Introduced by Dr. Arthur Burghes *Genome editing technologies are rapidly evolving and hold great promise for intervention in monogenic disease. The aim of this session is to provide an overview of established and emerging genome editing technologies, and their potential use in treatment of spinal muscular atrophy.* – Dr. Mandana Arbab "In vivo tissue delivery of antibody-antisense morpholino conjugates in a mouse model of spinal muscular atrophy" - Dr. Suzan Hammond "Cell Penetrating peptide-conjugated Morpholino: treatment for SMA symptomatic cases" - Dr. Monica Nizzardo "Myostatin inhibition improves functional outcome with SMN-restoring antisense oligonucleotide therapy in spinal muscular atrophy" - Dr. Haiyan Zhou

	"A transcriptomic-based drug repurposing strategy for the identification of new SMN-independent skeletal muscle treatments for spinal muscular atrophy "- Dr. Joseph Hoolachan "Identification of neuroprotective genes and drugs using a new C. elegans SMA model" - Dr. Elia Di Schiavi
15:15 - 15:45	*Coffee break-Main hall & Crick room (1st Floor)*
15:45 - 17:00	**Session 2 - Newborn screening & pre-symptomatic treatment** - Auditorium **Chaired by: Professor Wolfgang Müller-Felber,** Introduced by Professor Enrico Bertini "Lessons learned during 2 years of a "German pilot project for SMA-newborn screening" Prof. Wolfgang Müller-Felber "Nusinersen in infants who initiate treatment in a presymptomatic stage of spinal muscular atrophy (SMA): Interim results from the Phase 2 NURTURE study" – Professor Janbernd Kirschner "Onasemnogene Abeparvovec Gene-Replacement Therapy in Presymptomatic Spinal Muscular Atrophy: SPR1NT Study Update" – Professor Laurent Servais
17:00 - 18:00	**Workshop - Real - world data** - Auditorium **Chaired by Professor Jan Kirschner & Professor Nathalie Goemans,** *Approval of orphan drugs is often based on limited evidence. Collection of real-world data after approval is therefore indispensable to fill evidence gaps concerning safety and efficacy of new treatments. The workshop will review the current landscape and state-of-the-art of real-world data collection in SMA.*
17:00 - 18:00	**Poster session -Watson/Jacob/Kroll room (1st floor)**
18:00 - 20:00	*Wine and Cheese* sponsored by AFM TELETHON - Main hall

6th of February

08:00 - 09:30	**Session 3 – How SMN influences translation**- Auditorium **Chaired by: Dr. Gabriella Viero** Introduced by Professor Tom Gillingwater *The prevailing paradigm holds that SMN is primarily involved in housekeeping functions connected to snRNP assembly. In this session, we will expand this view and open a new scenario where SMN plays a crucial role at the crossroads of RNA metabolism and translational control of gene expression* - : Dr. Gabriella Viero "Identification of Functional SMNs and Suppressors of the Nonfunctional SMNE134K" Dr. Anton Blatnik "An impaired neurotrophic signaling hub drives motoneuron degeneration in spinal muscular atrophy" – Dr. Niko HENSEL "Stasimon contributes to the loss of sensory synapses and motor neuron death in a mouse model of spinal muscular atrophy" – Dr. Christian Simon "Proteomic profiling of human fibroblasts from patients with severe to mild types of spinal muscular atrophy" Dr. Sharon Owen
09:30 - 11:00	*Coffee break-Main hall & Crick room (1st floor)*
09:45 - 10:45	*Symposium: Understanding SMA from clinical trials to the real world – Biogen Auditorium*
11:00 - 12:30	**Session 4 – Symptomatic treatment**- Auditorium **Chaired by Professor Maryam Oskoui,** Introduced by Professor Laurent Servais *The therapeutic landscape in spinal muscular atrophy is rapidly evolving. The aim of this session is to provide an evidence-based overview of available pharmacological studies. The main tenants of risk of bias assessment and external validity will be reviewed to frame our interpretation of results.* – Professor Maryam Oskoui "Plasma Phosphorylated Neurofilament heavy chain levels over time in participants with infantile- and later-onset SMA: Data from the SHINE study : Professor Eugenio Mercuri

	"Onasemnogene Abeparvovec Gene-Replacement Therapy for spinal muscular atrophy Type 1: Pivotal Phase 3 Studies Clinical Update (STR1VE-EU and STR1VE-US)" – Professor Francesco Muntoni "SUNFISH Part 2: Efficacy and safety of risdiplam (RG7916) in patients with Type 2 or non-ambulant Type 3 spinal muscular atrophy (SMA)" - Professor Eugenio Mercuri "One-year follow up of treatment with intrathecal nusinersen and its effects on motor, respiratory and bulbar function in children with SMA types 1b-3a" – Dr. Feline Scheijmans
12:30 - 14:00	*Lunch Break-Main hall & Crick room (1st floor)*
12:45 - 13:45	*Symposium: Institute of Biotherapies - Auditorium* «Unique success path for basic science to large-scale bioproduction and patient access in the field of rare and neuromuscular diseases »
14:00 - 15:30	**Session 5 – SMN independent pathways**- Auditorium **Chaired by Professor Tom Gillingwater,** Introduced by Professor Kevin Talbot *Whilst SMN-targeted therapies are delivering life-changing treatments for SMA patients, they remain far from a cure. This session will explore SMN-independent pathways that can influence SMA across the lifespan, generating the opportunity for a second generation of 'SMN+' therapies*. – Professor Tom Gillingwater "Muscle regulates mTOR dependent axonal local translation in motor neurons via CTRP3 secretion: Implications for a neuromuscular disorder, spinal muscular atrophy" – Dr. Min Jeong Kye "Peripheral microvascular abnormalities and the endothelial cell autonomous defects secondary to SMN deficiency in spinal muscular atrophy" – Dr. Haiyan Zhou "Myopathy precedes neuronal phenotype in a new mild mousemodel in SMA" – Dr. Rashmi Kothary "Lamin A/C dysregulation contributes to cardiac pathology in a mouse model of severe spinal muscular atrophy" – Dr. Heidi Fuller
15:30 - 16:00	*Coffee break-Main hall & Crick room (1st floor)*
16:00 - 17:00	**Workshop – New phenotypes in SMA** - Auditorium **Chaired by Professor Eugenio Mercuri with Marion Main and Valery Sansone** New phenotypes in infants (type 1) - Marion Main (London) New phenotypes in children (type 2) - Eugenio Mercuri (Rome) New phenotypes in adults - Valeria Sansone (Milan)

17:00 - 18:00	**Poster session - Watson/Jacob/Kroll room (1st floor)**
19:00	*GALA DINNER*

7th of February

08:00 - 09:30	**Session 6 – Comprehensive management of SMA**- Auditorium **Chaired by Professor Ludo van der Pol** Introduced by Professor Stephania Corti Title tbd Professor Ludo van der Pol "Respiratory muscle strength in spinal muscular atrophy" – Dr. Esther Veldhoen "Mastication problems in spinal muscular atrophy type II and III" – Dr. Marise van der Heul "Development of a predictive body fat equation for spinal muscular atrophy type I children" – Dr. Simona Bertoli "SMA treatment and trials: new horizons for migration phenomenon to Italy "- Chiara Mastella
09:30 - 11:00	*Coffee break-Main hall & Crick room (1st floor)*
09:45 - 10:45	*Symposium: Avexis:* The Coming of Age of Gene Therapy for SMA *Auditorium*
11:00 - 12:30	**Session 7 – The role of biomarkers in SMA**- Auditorium **Chaired by: Professor Kathryn Swoboda** Introduced by Professor Ludo van der Pol Title tbd : : Professor Kathryn Swoboda "Development of motor function and changes in NFL in CSF in children with SMA treated with nusinersen" Prof. Mar Tulinus "Magnetic Resonance Imaging of skeletal muscle in a cross-sectional cohort of patients with Spinal Muscular Atrophy type 2-3" Dr. Louise Otto "pNfH is a reliable biomarker for adult SMA patients' follow-up upon nusinersen treatment" Dr. Piera Smeriglio In vivo upper arm ATP metabolism during dynamic exercise in SMA type 3 and 4 – Dr. L.E. Habets

12:30 - 13:30	**Closing talk :Outstanding questions for SMA research in a new therapeutic era** **Professor Kevin Talbot & Professor Laurent Servais** Dramatic therapeutic advances in SMA have been achieved despite the fact that some major aspects of pathogenesis remain unexplained. This session will summarise the highlights of the conference and review the key scientific questions which remain to be resolved and how these inform the future of SMA therapeutics. - Professor Kevin Talbot & Professor Laurent Servais
13:30 - 14:00	**Closing Ceremony & Awards**- Auditorium **Professor Kevin Talbot & Mencia de Lemus**
14:00 - 15:30	*Lunch Break* -Main hall & Crick room (1st floor)