



5th

International Scientific Congress on
Spinal Muscular Atrophy

BUDAPEST

11th — 14th March 2026

PROGRAMME



WEDNESDAY 11TH MARCH 2026

08.30 - 12.30	Global SMAAdvocacy Event (Separate event from the Congress, per invitation only)
12.30 - 14.00	Registration
14.00 - 15.30	Workshops (running in parallel) Assessing fatigability in Spinal Muscular Atrophy: Exploring the contribution of neuromuscular junction dysfunction Organised by the Industry & Jacqueline Montes, Columbia University Irving Medical Center, New York, USA Beyond boundaries: Elevating SMA care for the future Organised by the Industry Old signals, new insights: Electrophysiology as a biomarker in SMA Organised by Kathryn Swoboda, Massachusetts General Hospital, Boston, MA, USA & Renske Wadman, UMC Utrecht, The Netherlands MAPS - Mobility, Activity, Play, and Sports Organised by José Longatto, Great Ormond Street Hospital, London, United Kingdom Respiratory care in SMA Organised by Esther Veldhoen, UMC Utrecht, The Netherlands Nutritional management in SMA in the new treatment era: From fundamental science to clinical practice Organised by Simona Bertoli, IRCCS Istituto Auxologico Italiano, Milan, Italy
15.30 - 16.00	Coffee Break
16.00 - 17.30	Workshops (running in parallel) Every motor neuron matters - Biomarkers and treatment choice in SMA Organised by the Industry & Eduardo Tizzano, Vall D'hebron Research Institute (VHIR), Barcelona, Spain Contractures in SMA: From scientific insights to therapeutic targets Organised by FundAME, Madrid, Spain Addressing unmet Needs in SMA care through multidisciplinary collaboration and education Organised by the Industry The role of early parental empowerment in management of emergencies and urgencies at home in pharmacologically treated SMA type 1 Organised by Chiara Mastella, SAPRE-UONPIA, Fondazione IRCCS Cà Granda Ospedale Maggiore Policlinico Milan, Italy SMA and neurodevelopmental disorders: A call for harmonized strategies Organised by Giovanni Baranello, UCL Great Ormond Street Institute of Child Health, London, United Kingdom Bench to bedside and back - Fundamental discoveries in connection with patient journeys Organised by Ewout Groen, UMC Utrecht, The Netherlands
19.00 - 21.00	Welcome Reception

THURSDAY 12TH MARCH 2026

08.00 - 10.00	Registration & Welcome Coffee
10.00 - 10.30	Welcome and Opening Talk Tom Gillingwater, Chair Scientific Advisory Board SMA Europe Yasemin Erbas, President SMA Europe Nicole Gusset, CEO SMA Europe
10.30 - 12.15	Session 1: Biomarkers in SMA Chair: Ewout Groen UMC Utrecht, The Netherlands Defining and refining biomarkers in Spinal Muscular Atrophy Keynote Speaker: Ewout Groen, UMC Utrecht, The Netherlands Muscle-specific extracellular vesicles: A novel biomarker for Spinal Muscular Atrophy Melissa Bowerman, Keele University, United Kingdom Therapeutic and biomolecular effects of long-term nusinersen treatment in 5q Spinal Muscular Atrophy type 2 and 3: A long-term CSF proteomic study Gina Cebulla, Heidelberg University Hospital, Germany Motor unit patterns correlate with severity in symptomatic patients with Spinal Muscular Atrophy Renske Wadman, UMC Utrecht, The Netherlands Multi-omic Biomarkers for personalized Spinal Muscular Atrophy management: A multicenter Italian study Stefania Corti, Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Università degli Studi di Milano, Italy Titin and the muscle-brain axis in SMA: A promising biomarker for Nusinersen therapy across CSF transcriptome and radiological perspective Francesca Dragoni, IRCCS Mondino Foundation, Italy
12.30 - 14.00	Lunch Break
13.00 - 13.45	Industry Sponsored Symposium
14.00 - 15.45	Session 2: Regeneration and disease modifiers Chair: Linda Greensmith, University College London, United Kingdom A novel optogenetic cell therapy approach to restore muscle innervation in ALS Keynote speaker: Linda Greensmith, University College London, United Kingdom Biomimetic viscoelastic hydrogels promote motor neuron outgrowth and modulate Astrocyte behaviour for Spinal Muscular Atrophy regeneration applications Cian O'Connor, Harvard University and Royal College of Surgeons in Ireland, Ireland

THURSDAY 12TH MARCH 2026

	Neuromuscular organoids reveal developmental transcriptomic dysregulation in SMA Morgan Gazzola, I-Stem, Corbeil-Essonnes, France A novel mouse model of X-linked Spinal Muscular Atrophy offers a platform for preclinical development of UBA1 targeting therapeutics Hannah Crick, University of Edinburgh, United Kingdom Gene Modifiers of Spinal Muscular Atrophy Umrao Monani, Columbia University Medical Center, USA Significant microvascular pathology is driven by specific SMN depletion in endothelial cells: The 'EndoSMA' mouse Alaa Alshwayyat, University of Aberdeen, United Kingdom
15.45 - 16.15	Flash Poster Presentations Proteomic profiling of cytoplasmic stress-induced liquid condensates of SMN Oliver Gruss, Institute of Genetics, University of Bonn, Germany Exploring the trajectory of swallowing within psychomotor development in Spinal Muscular Atrophy: Moving toward integrated care Sofia Gandolfi, Fondazione IRCCS Istituto Neurologico Carlo Besta, Milan, Italy 5q-SMA emergency management support tool: Collaborative construction of an emergency card Aline Giuliani, Viva Iris, Uberlândia, Brazil Polysomnographic findings in children with Spinal Muscular Atrophy Milena Villamil, Osorio Fundación Hospital Pediátrico la Misericordia, Bogotá, Colombia Prenatal SMN-dependent defects in translation uncover reversible primary cilia phenotypes in SMA Helena Chaytow, University of Edinburgh, United Kingdom Posttranslational regulation of the Survival Motor Neuron (SMN) complex Claudia Cobos, University of Vienna, Institute of Biological Chemistry, Austria
16.15 - 16.45	Coffee Break & Poster Session
16.15 - 17.30	Poster Session 1
17.30 - 18.15	Industry Sponsored Symposium
19.30 - 22.00	<i>Thank you Dinner (per invitation only)</i>

FRIDAY 13TH MARCH 2026

08.00 - 08.45	Industry Sponsored Symposium
09.00 - 10.30	Session 3: Metabolism and diet in SMA Chair: Alessandro Usiello, University of Campania "Luigi Vanvitelli", Italy Mitochondrial dysfunction in Spinal Muscular Atrophy: emerging insights from disease mechanisms to therapeutic targets Camilla Bean, UniCamillus, Rome, Italy Adipokines in adult patients with Spinal Muscular Atrophy Type 3: Challenging the canonical narrative Marija Miletic, University Clinical Center of Serbia, Serbia Oxidative stress in Spinal Muscular Atrophy (SMA): Cellular Mechanisms and antioxidant therapeutic potential Phoebe Rassinoux, INMG-PGNM, Lyon, France Mitochondrial and redox perturbations in SMA: Insights from multi-organ omics and therapeutic targeting of the NRF2-KEAP1 Axis Sofia Vrettou, Center for Molecular Medicine, University of Cologne, Germany SMN protein levels impact the metabolism of neuroactive amino acids in preclinical models and SMA patients Keynote Speaker: Alessandro Usiello, University of Campania "Luigi Vanvitelli", Italy
10.30 - 11.00	Coffee Break
11.00 - 12.30	Session 4: Sensorimotor function and fatiguability in SMA Chair: George Mentis, Columbia University, New York, USA Sensory-motor neuronal dysfunction: An essential pathomechanism in animal models and patients of SMA Keynote Speaker: George Mentis, Columbia University, New York, USA Cortical GABAergic dysregulation and metabolic alterations in a Spinal Muscular Atrophy mouse model Giovanna Menduti, Neuroscience Institute Cavalieri Ottolenghi, University of Turin, Italy Advancing assessment of perceived physical fatigability using the SMA EFFORT Rafael Rodriguez-Torres, Columbia University Irving Medical Center, USA smn-1 regulates motoneurons and dopaminergic neurons crosstalk in C. elegans Giada Onorato, Institute of Biosciences and BioResources, CNR, Naples, Italy Motor neuron pathology drives spinal circuit defects and phenotype of Spinal Muscular Atrophy with respiratory distress type 1 Gabriel Kohn, Carl-Ludwig-Institute for Physiology, Leipzig University, Germany

FRIDAY 13TH MARCH 2026

12.30 - 14.00	Lunch Break
13.00 - 13.45	Industry Sponsored Symposium
14.00 - 15.45	Session 5: Optimizing treatments for SMA Chair & Keynote Speaker: Richard Finkel, St Jude Children's Research Hospital, Memphis, TN, USA Rescuing translation defects as a new SMN-independent therapeutic strategy for Spinal Muscular Atrophy Ilaria Bruno, Institute of Biophysics, CNR, Trento, Italy Respiratory function in adults with Spinal Muscular Atrophy type 1 and 2 treated with risdiplam Femke Vercoelen, UMC Utrecht, The Netherlands Synthetic XNA/DNA-Based Antisense Oligonucleotides Enhance SMN2 Gene Expression in Spinal Muscular Atrophy Cell Models Beyza Aydin, T.C. Üsküdar University, Turkey Arrested motor axon development drives temporal requirement for SMN restoration in severe SMA Charlotte Sumner, Johns Hopkins University School of Medicine, Baltimore, MD, USA Muscle-specific kinase agonist antibody, ARGX-119, coadministered with an SMN2 splice modulator improves muscle strength and voluntary locomotion in a mouse model of Spinal Muscular Atrophy Rani Coppejans, ArgenX, Belgium
15.45 - 16.15	Flash Poster Presentations Muscle-specific Smn depletion does not recapitulate the metabolic defects observed in spinal muscular atrophy mice Luis Felipe Velandia Dorta, Keele University, United Kingdom Beyond spinal motor neurons: cortical projection neuron numerical and morphological alterations reveal selective vulnerability to SMN loss also in the brain Roberta Schellino, University of Turin and Neuroscience Institute Cavalieri Ottolenghi, Italy Anesthetic risks during intrathecal nusinersen admission in children with Spinal Muscular Atrophy type 1 Femke Vercoelen, UMC Utrecht, The Netherlands Switching DMT in SMA pediatric population: The French experience Marta Gomez, Raymond Poincare Hospital, Garches, France The Natural History of SMA with Four SMN2 Copies: Evidence from the Spanish Registry (CUIDAME) Karolina Aragon Gawinska Hospital Universitari i Politècnic La Fe, Valencia, Spain

FRIDAY 13TH MARCH 2026

	Management and aspects of pregnancy of SMA on Nusinersen treatment - Case series Maria Judit Molnar, Institute of Genomic Medicine and Rare Disorders, Budapest, Hungary
16.15 - 16.45	Coffee Break & Poster Session
16.15 - 17.30	Poster Session 2
17.30 - 18.30	<i>Early Stage Researchers (ESRs) Mentoring Session (by invitation only)</i>
19.30 - 23.00	Gala Dinner with Community Awards

SATURDAY 14TH MARCH 2026

08.30 - 10.00

Session 6: New standards of care for SMA

Chair: Andreas Ziegler, Heidelberg University, Germany

Times are still changing - How to screen and whom to treat with how many therapies

Keynote Speaker: Andreas Ziegler, Heidelberg University, Germany

Ensuring best hip management in individuals with SMA across the UK

Giovanni Baranello, Translational and Clinical Research Institute, Newcastle University UK SMA Care, United Kingdom

Immune and hematopoietic defects in SMA are rescued by SMN repletion

Paula Guillamón, Gil Universitat de Lleida, Spain

Novel SMN1 variants identified by false positive SMA newborn screening test: Therapeutic hurdles and functional solutions

Brunhilde Wirth, Institute of Human Genetics, Univ. Cologne, Germany

Elective preterm birth as a strategy for early intervention in Spinal Muscular Atrophy with two SMN2 copies

Tamara Dangouloff, University Hospital Liege, Belgium

10.00 - 10.30

Coffee Break

10.30 - 12.00

Session 7: Cognitive function in SMA

Chair: Susana Quijano-Roy, Raymond Poincaré University Hospital (UVSQ), Garches, France

Neurodevelopmental phenotype in SMA type 1: Proposal of an evaluation for routine clinical practice

Keynote Speaker: Susana Quijano-Roy, Raymond Poincaré University Hospital (UVSQ), Garches, France

Cognitive development in children with 5q-SMA identified by neonatal screening – 4 years follow-up

Heike Kölbel, Universitätsmedizin Essen, Germany

Cerebellar pathology contributes to motor and cognitive deficits in Spinal Muscular Atrophy

Florian Gerstner, University of Leipzig, Carl-Ludwig-Institute for Physiology, Germany

Prenatal intervention in an SMA mouse model ameliorates neurodevelopmental disorders associated with spinal muscular atrophy

Charalambos Demetriou, University College London, United Kingdom

SMACK! Project: Impact of the parent-child interaction in the early neurodevelopmental stages of a cohort of children with SMA diagnosed by neonatal screening – A pilot study

Stefano Parravicini Fondazione, IRCCS Istituto Neurologico Besta, Milan, Italy

SATURDAY 14TH MARCH 2026

12.00 - 12.30

Late Breaking News

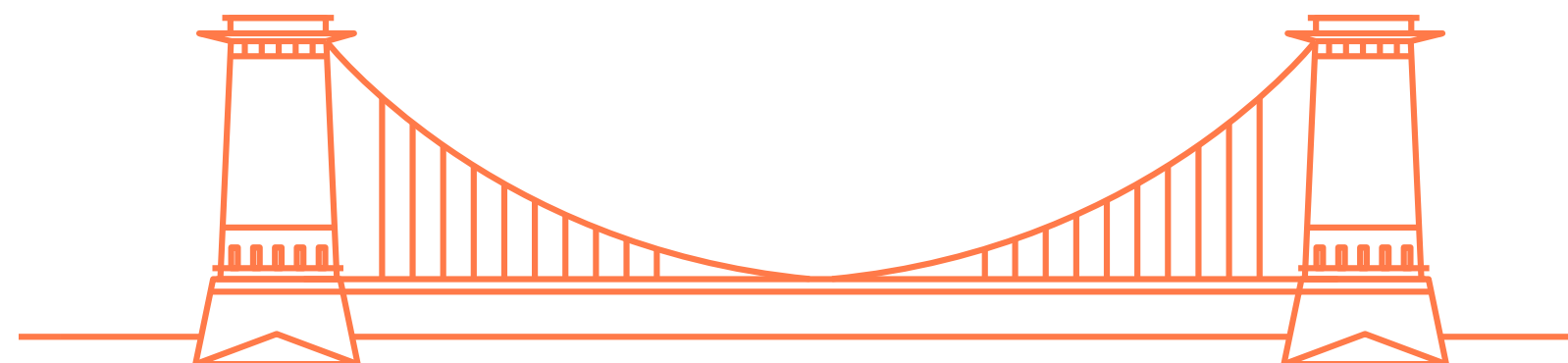
12.30 - 13.00

Closing & Celebrations

Scientific Poster Awards

13.00 - 13.30

Grab a lunch box in the exhibition area



**SMA
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