

**21st INTERNATIONAL CONGRESS OF THE
WORLD MUSCLE SOCIETY**

October 4-8, 2016
Granada, Spain

Session Type	Oral Presentation
Session Title	Late Breaking Session
Date	Saturday, October 8, 2016
Time	14:00 – 16:00 CEST
Title	<u>Nusinersen treatment of infantile-onset spinal muscular atrophy (SMA): study design and initial interim efficacy and safety findings from the phase 3 ENDEAR study</u> <i>Kuntz N,¹ Farwell W,² Zhong ZJ,² Sun P,² Gheuens S,² Schneider E,³ and Finkel R⁴ on behalf of the ENDEAR study group</i> <i>¹ Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, IL, USA; ² Biogen, Cambridge, MA, USA; ³ Ionis Pharmaceuticals Inc., Carlsbad, CA, USA; ⁴ Division of Neurology, Department of Pediatrics, Nemours Children's Hospital, Orlando, FL, USA</i>

Session Type Oral Presentation
Session Title Late Breaking Session
Date **Saturday, October 8, 2016**
Time **14:00 – 16:00 CEST**
Title **[Nusinersen in Pre-symptomatic Infants With Spinal Muscular Atrophy \(SMA\): Interim Efficacy and Safety Results From the Phase 2 NURTURE Study](#)**
Bertini E,¹ Hwu W-L,² Reyna SP,³ Farwell W,³ Gheuens S,³ Sun P,³ Zhong ZJ,³ Su J,⁴ Schneider E,⁴ De Vivo DC,⁵ on behalf of the NURTURE study investigators
¹Unit of Neuromuscular and Neurodegenerative Disorders, Post-Graduate Bambino Gesù Children's Research Hospital, Rome, Italy; ²Department of Medical Genetics and Pediatrics, National Taiwan University Hospital, Taipei, Taiwan; ³Biogen, Cambridge, MA, USA; ⁴Ionis Pharmaceuticals, Inc., Carlsbad, CA, USA; ⁵Department of Neurology, Columbia University Medical Center, New York, NY, USA

Session Type Oral Presentation
Session Title Late Breaking Session
Date **Saturday, October 8, 2016**
Time **14:00 – 16:00 CEST**
Title **[Nusinersen in treatment-naïve patients with later-onset spinal muscular atrophy \(SMA\): efficacy results from a phase 1b/2a multicentre study \(CS2\) and its open-label extension \(CS12\)](#)**
Darras BT,¹ Chiriboga CA,² Montes J,² Swoboda KJ,³ Johnson N,⁴ Iannaccone ST,⁵ Castro D,⁵ Mignon L,⁶ Norris D,⁶ Gamelin L,⁶ Xia S,⁶ Fine S,⁶ Bennett CF,⁶ Bishop K,⁶ Foster R,⁷ Gheuens S,⁷ Sun P,⁷ Farwell W,⁷ Schneider E,⁶ De Vivo DC,² on behalf of the CS2 and CS12 study investigators
¹Department of Neurology, Boston Children's Hospital, Boston, MA, USA; ²Columbia University Medical Center, New York, NY, USA; ³Massachusetts General Hospital; Department of Neurology, Boston, MA, USA; ⁴Neurology, University of Utah School of Medicine, Salt Lake City, UT, USA; ⁵Pediatrics, University of Texas Southwestern Medical Center, Dallas, TX, USA; ⁶Ionis Pharmaceuticals, Inc., Carlsbad, CA, USA; ⁷Biogen, Cambridge, MA, USA

Session Type Poster Presentation
Session Title Poster Presentations 1: Parallel Sessions
Date **Wednesday, October 5, 2016**
Time **14:30 – 16:00 CEST**
Poster Number 45
Title **[The Natural History of Type 1 Spinal Muscular Atrophy in Taiwan](#)**
Ou S-F,¹ Jong Y-J,² Ho C-S,³ Lee W-T,⁴ Lin K-L,⁵ Jones CC,⁶ on behalf of the SMA Study Group
¹Antai Medical Care Cooperation Antai Tian-Sheng Memorial Hospital, Pingtung, Taiwan; ²Kaohsiung Medical University Hospital, Kaohsiung; National Chiao Tung University, Hsinchu, Taiwan; ³MacKay Children's Hospital, Taipei, Taiwan; ⁴National Taiwan University Hospital, Taipei, Taiwan; ⁵Chang Gung Children's Hospital and Chang Gung Memorial Hospital, Linkou, Taiwan; ⁶Biogen, Cambridge, MA, USA

Session Type Poster Presentation
Session Title Poster Presentations 1: Parallel Sessions
Date **Wednesday, October 5, 2016**
Time **14:30 – 16:00 CEST**
Poster Number 46
Title **[SMA Subtype Concordance in Siblings: Findings From the Cure SMA Cohort](#)**
Jones CC,¹ Cook SF,¹ Hobby K,² Jarecki J²
¹Biogen, Cambridge, MA, USA; ²Cure SMA, Elk Grove Village, IL, USA



Session Type	Poster Presentation
Session Title	Poster Presentations 1: Parallel Sessions
Date	Wednesday, October 6, 2016
Time	14:30 – 16:00 CEST
Poster Number	47
Title	<u><i>Awareness and Utilization of the Hammersmith Functional Motor Scale – Expanded (HFMSE): A Survey</i></u> Yon J, ¹ Babrowicz J, ² Montes J ³ ¹ Biogen, Cambridge, MA, USA; ² Nielsen, New York, NY, USA; ³ Departments of Neurology and Rehabilitation and Regenerative Medicine, Columbia University, New York, NY, USA
