



**AMERICAN ACADEMY OF NEUROLOGY
66TH ANNUAL MEETING
APRIL 26-MAY 3, 2014
PHILADELPHIA, PA**

Session Type Platform Presentation
Session Title S6 – Neuromuscular Disease: Gene Therapy
Date Tuesday, April 29, 2014
Time 1:30 p.m. – 1:45 p.m. ET
Presentation Number S6.003
Presentation **RESULTS OF A PHASE 2 OPEN-LABEL STUDY OF ISIS-SMN_{RX} IN PATIENTS WITH INFANTILE (TYPE 1) SPINAL MUSCULAR ATROPHY**
Richard Finkel,¹ John Day,² Claudia Chiriboga,³ Jiri Vajsar,⁴ Dawn Cook,¹ Karolina Watson,² Shirley Paulose,² Rosangel Cruz,³ Jacqueline Montes,³ Darryl De Vivo,³ Mason Yamashita,⁵ Dawn McGuire,⁵ Katie Alexander,⁵ Daniel Norris,⁵ Frank Bennett,⁵ Kathie Bishop⁵
¹Orlando, FL, USA, ²Stanford, CA, USA, ³New York, NY, USA, ⁴Toronto, ON, Canada, ⁵Carlsbad, CA, USA

Session Type Poster and Oral Presentations
Session Title Emerging Science
Date Wednesday, April 30, 2014
Time 6:15 p.m. – 7:30 p.m. ET (Poster)
6:30 p.m. – 6:33 p.m. ET (Data Blitz Presentation)
Poster Number 006
Presentation **RESULTS OF A PHASE 2 STUDY OF ISIS-SMN_{RX} IN CHILDREN WITH SPINAL MUSCULAR ATROPHY**
Chiriboga, Claudia¹; Swoboda, Kathryn²; Darras, Basil³; Iannaccone, Susan⁴; Montes, Jacqueline¹; Castro, Diana⁴; Holuba, Nicole¹; Rausch, Nicole²; Visyak, Nicole³; Dunaway, Sally¹; Trussell Donata²; Pasternak, Amy³; Neilson, Leslie⁴; De

Vivo, Darryl¹; McGuire, Dawn; Norris, Dan⁵; Alexander, Katie⁵; Bennett, C. Frank⁵; Bishop, Kathie⁵

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³ Boston Children's Hospital; ⁴ UT Southwestern Medical Center;

⁵ Isis Pharmaceuticals, Inc.

Session Type Oral Presentation
Session Title Presidential Plenary Session
Date **Tuesday, April 29, 2014**
Time **9:00 a.m. – 12:00 p.m. ET**
Presentation **DEGENERATIVE ATAXIAS: FROM GENES TO THERAPIES**
Stefan Pulst
Salt Lake City, UT, USA

Session Type Platform Presentation
Session Title S47 – Movement Disorders: Huntington's Disease, Ataxia, and Wilson's Disease
Date **Thursday, May 1, 2014**
Time **2:15 p.m. – 2:30 a.m. ET**
Poster Number **S47.006**
Presentation **TREATMENT OF SPINOCEREBELLAR ATAXIA TYPE 2 (SCA2) WITH MOE ANTISENSE OLIGONUCLEOTIDES**
Daniel R. Scoles,¹ Gene Hung,² Lance T. Pflieger,¹ Khanh K. Thai,¹ Frank Bennett,² Stefan Pulst¹
¹Salt Lake City, UT, USA
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