



ISIS[®]
PHARMACEUTICALS

**11TH ANNUAL MEETING OF THE
OLIGONUCLEOTIDE THERAPEUTIC SOCIETY**

**OCTOBER 11-14, 2015
LEIDEN, NETHERLANDS**

Session Type Oral Presentation
Session Title Exon Skipping
Date **October 12, 2015**
Time **11:15 a.m. – 11:45 a.m.**
Session Number Session II
Title **ISIS-SMN_{RX} BACKGROUND AND CURRENT STATUS**
C. Frank Bennett
Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Oral Presentation
Session Title Exon Skipping
Date **October 12, 2015**
Time **11:45 a.m. – 12:00 p.m.**
Session Number Session II
Title **ANTISENSE-OLIGONUCLEOTIDE-DIRECTED INHIBITION OF
NONSENSE-MEDIATED mRNA DECAY**
Tomoki Nomakuchi^{1,2}, Frank Rigo³, Isabel Aznarez¹, and Adrian R. Krainer¹
1 Cold Spring Harbor Laboratory, Cold Spring Harbor, New York, USA.
2 Stony Brook University School of Medicine, Stony Brook, New York, USA.
3 Isis Pharmaceuticals, Carlsbad, California, USA.

Session Type Oral Presentation
Session Title Emerging Concepts in RNA Biology
Date **October 12, 2015**
Time **3:45 p.m. – 4:00 p.m.**
Session Number Session III
Title **ANTISENSE OLIGONUCLEOTIDE-MEDIATED MAPT mRNA SPLICING REVEALS A TOXIC TAU ISOFORM IN MOUSE MODELS OF TAUOPATHY**
Kathleen M. Schoch¹, Sarah L. DeVos¹, Carey S. Kebodeaux¹, Rebecca L. Miller¹, Seung J. Chun², Michaela Norrbom², David F. Wozniak³, Hana N. Dawson⁴, C. Frank Bennett², Frank Rigo², Timothy M. Miller¹
1 Neurology, Hope Center for Neurological Disorders, Washington University in St. Louis, MO, 2 Isis Pharmaceuticals, Carlsbad, CA, 3 Psychiatry, Washington University in St. Louis, MO, 4 Neurology, Duke University Medical Center, Durham, NC

Session Type Poster Presentation
Date **October 12, 2015**
Time **4:30 p.m. - 6:30 p.m.**
Session Number Poster Session I
Title **TREATMENT OPPORTUNITIES FOR CYSTIC FIBROSIS USING AEROSOLIZED ANTISENSE OLIGONUCLEOTIDES**
Shuling Guo, Jeff Crosby, Chenguang Zhao, Michael McCaleb, and Brett P. Monia
Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Oral Presentation
Session Title Oligonucleotide Safety
Date **October 13, 2015**
Time **1:20 p.m. - 1:55 p.m.**
Session Number Session V
Title **A MECHANISM OF HEPATOTOXICITY FOR HIGH AFFINITY ANTISENSE OLIGONUCLEOTIDES**
Sebastien Burel
ISIS Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Oral Presentation
Session Title Early Drug Discovery
Date **October 13, 2015**
Time **3:45 p.m. - 4:15 p.m.**
Session Number Session VI
Title **ALLELE SELECTIVE SUPPRESSION OF MUTANT HUNTINGTIN BY SNP TARGETING ANTISENSE OLIGONUCLEOTIDE**
Punit Seth
Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Oral Presentation
Session Title Early Drug Discovery
Date **October 13, 2015**
Time **5:15 p.m. - 5:30 p.m.**
Session Number Session VI
Title **ANTISENSE OLIGONUCLEOTIDES FOR SOD1 IMPROVE FUNCTION AND EXTEND LIFE OF SOD1-G93A MICE**
Alexander McCampbell¹, Linhong Sun¹, Yi Luo¹, Nicole Comfort¹, Jessica Goodman¹, Mingdi Zhang¹, Brandon Farley¹, Tracy Cole², Holly Kordasiewicz², Eric Swayze², Frank Bennett²
¹Biogen, Cambridge, MA; ² Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Poster Presentation
Date **October 13, 2015**
Time **5:30 p.m. - 7:30 p.m.**
Session Number Poster Session II
Title **COMPARISON OF RENAL ANTISENSE OLIGONUCLEOTIDE ACTIVITY AND DISTRIBUTION IN MODELS OF ACUTE AND CHRONIC KIDNEY DISEASE**
Alex Bell, Aaron Donner, Mark Graham, Gene Hung, Donna Sipe, Xiaokun Xiao, Bea DeBrosse-Serra, Stan Riney, and Rosanne Crooke
Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Poster Presentation
Date **October 13, 2015**
Time **5:30 p.m. - 7:30 p.m.**
Session Number Poster Session II
Title **ATAXIN-3 EXON SKIPPING AS A TREATMENT STRATEGY FOR SPINOCEREBELLAR ATAXIA TYPE 3**
Lodewijk J.A. Toonen¹, Melvin M. Evers¹, Frank Rigo², and Willeke M.C. van Roon-Mom¹
1 Department of Human Genetics, Leiden University Medical Center, Leiden, The Netherlands. 2 Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type Poster Presentation
Date **October 13, 2015**
Time **5:30 p.m. - 7:30 p.m.**
Session Number Poster Session II
Title **IDENTIFICATION OF A HUMAN CLINICAL CANDIDATE ASO SELECTIVELY TARGETING THE P23H VARIANT OF RHODOPSIN FOR THE TREATMENT OF P23H RHODOPSIN-MEDIATED ADRP**
Sue F. Murray¹, Ali Jazayeri¹, Raechel Peralta¹, Andy Watt¹, Timothy A. Vickers¹, Shuling Guo¹, Scott Henry¹, Peter Adamson², Punit P. Seth¹, Brett P. Monia¹, Michael L. McCaleb¹
*1 Isis Pharmaceuticals, Inc., Carlsbad, CA, USA.
2 Ophthalmology, Discovery Performance Unit, GlaxoSmithKline, Stevenage, UK*

Session Type Oral Presentation
Session Title Awards Presentations & Talks
Date **October 14, 2015**
Time **11:20 p.m. - 11:40 p.m.**
Session Number Session VIII
Title **ANTISENSE OLIGONUCLEOTIDE THERAPY FOR ALS AND FRONTAL TEMPORAL DEMENTIA CAUSED BY GAIN OF TOXICITY FROM HEXANUCLEOTIDE EXPANSION IN THE C9ORF72 GENE**
Frank Rigo
ISIS Pharmaceuticals, Inc., Carlsbad, CA, USA.

Session Type	Oral Presentation
Session Title	Clinical Studies
Date	October 14, 2015
Time	3:00 p.m. - 3:30 p.m.
Session Number	Session IX
Title	EFFECTIVE ANTISENSE TARGETING OF APOC-III IN FAMILIAL CHYLOMICRONEMIA SYNDROME REVEALS NOVEL BIOLOGY AND THERAPY
	<i>Joe Witztum</i> <i>University of California, San Diego</i>
