Industry-Sponsored Satellite Sessions

Industry Sponsored Satellite Sessions are independently staged and accredited or non-accredited educational or product theater events. A gateway fee making them accessible to attendees is paid by the presenters.

LIVE IN BOSTON:

Advancing the Care of a Pediatric Neurotransmitter Disorder: Experience with an Investigational Intraputaminal Gene Replacement Therapy for the Treatment of AADC Deficiency

Thursday, September 30, 7:00 AM - 8:15 AM, Rm 312

Please join our expert faculty for an educational symposium at the 50th Child Neurology Society (CNS) Annual Meeting as they:

- Provide an overview of aromatic L-amino acid decarboxylase (AADC) deficiency and the eladocagene exuparvovec construct
- Review clinical experience with treatment options in development for AADC deficiency
- Provide rationale for the putamen as a target delivery site for central nervous system gene therapy
- Discuss the real-world experience of treating patients with investigational eladocagene exuparvovec

Dr. Ashutosh Kumar
Assistant Professor, Pediatrics and Neurology, Penn State Health Milton S. Hershey Medical Center

Dr. Daniel J. Curry
Director, Functional Neurosurgery & Epilepsy Surgery, Texas Children's Hospital

Prof. Agathe Roubertie, Department of Pediatric Neurology, Hôpital Gui de Chauliac, Montpellier, France

Dr. Matthew Klein, Chair, Chief Development Officer, PTC Therapeutics

Pre-register: https://web.cvent.com/event/df74d1be-e6db-4d59-9463-5889eedf57ae/regProcessStep1?locale=en-US&tm=wyyg03xGEf5WixXG2AlbAMRpQXO-VmEnQv2X9P7T0Nk

Mariya Elterman, melterman@ptcbio.com

Financial support by PTC Therapeutics

LIVE IN BOSTON:

Practical Clinical Management of Dravet Syndrome: Reconsidering the Standard of Care

Thursday, September 30, 12:00 PM - 2:00 PM, Rm 312

Dravet syndrome (DS) is a debilitating, epileptic encephalopathy of childhood for which few treatment options were available in the United States prior to 2018. Because, until recently, treatment options were limited, most patients retained a high seizure burden even with polypharmacy, with little positive impact on non-seizure-related outcomes. Novel treatment options, however, provide an unprecedented level of seizure control with prolonged intervals of seizure freedom and ≥75% seizure reduction in as many as 50% of patients, with evidence emerging that the robust reduction in seizure frequency also improves cognitive outcomes. Future investigations will show if treatment with novel agents can translate into DS patients having a greater likelihood of better long-term neurodevelopmental outcomes. This symposium will review DS and its clinical diagnosis and management, with a practical focus on rational therapy choices that optimize patient management and long-term outcomes.

AGENDA

Noon – 12:30 PM – On-site Check-in and Lunch

12:30 PM – 12:35 PM – Introduction
Elizabeth A. Thiele, MD, PhD (Program Chair)
Harvard Medical School
Massachusetts General Hospital

12:35 PM – 12:50 PM – Dravet Syndrome: Clinical Updates and Therapeutic Options
Elizabeth A. Thiele, MD, PhD

12:50 PM – 1:15 PM – Seizure Management: From Clinical Trials to Clinical Care
Elaine C. Wirrell, MD, FRCPC
Mayo Clinic

1:15 PM – 1:35 PM – Secondary Outcomes: Real-World Perspectives on Long-Term Outcomes and Clinical Expectations
Joseph E. Sullivan, MD
University of California, San Francisco (UCSF)
UCSF Benioff Children's Hospital
Pediatric Epilepsy Center of Excellence

1:35 PM – 2:00 PM – Q&A

Pre-register: www.millermeded.com/dravet
Kerri Leonard, kerri.leonard@millermeded.com

Financial support by Zogenix

LIVE IN BOSTON:

Looking Back to Move Forward: Demonstrating the Potential Benefits of Early Treatment with SPINRAZA

Friday, October 1, 7:00 AM - 8:15 AM, Rm 312

Over the past 5 years, the spinal muscular atrophy (SMA) field has been transformed by the approval of disease-modifying therapy, resulting in evolving patient phenotypes and treatment expectations. Pull up an armchair and join us for an intimate fireside chat as our experts, Drs Crystal Proud and Julie Parsons, lead us on a journey through time exploring different eras of SMA treatment including the era prior to treatment availability, the early years following approval of SPINRAZA® (nusinersen), and SMA today.

Crystal Proud, MD
Neuromuscular Neurologist
Managing SMA patients since 2014
Children's Hospital of the King's Daughters
Norfolk, VA

Julie Parsons, MD
Pediatric Neurologist
Managing SMA patients since 2007
University of Colorado School of Medicine and
Children's Hospital Colorado
Aurora, CO

Kelly Fay, kelly.fay@biogen.com

Financial support by Biogen

VIRTUAL:

Industry Sponsored Product Theater Spinal Muscular Atrophy (Novartis Gene Therapies)

Tuesday, September 28, 1:00 PM EDT

Monday, October 4, 1:00 PM EDT

Dr. Sandra Reyna, VP of Global Medical Affairs and is the Head of Therapeutic Area at Novartis Gene Therapies.

Catherine Glaub, SMA Community Member, and a parent of a child with SMA

http://towermedicalmedia.com/livestream

Financial support by Novartis

Session links provided on virtual platform and on CNS website 2021 Annual Meeting page:
https://www.childneurologysociety.org/colleagues/network/cns-annual-meeting/

50TH CNS ANNUAL MEETING
PAST•PRESENT•FUTURE

Industry-Sponsored Seminars

VIRTUAL:

A Targeted Approach to Taming NF1-Associated Tumors: Navigating the Child Neurologist's Role in an Evolving Treatment Calculus

Monday, October 4, 7:00 PM EDT

This educational activity is targeted to pediatric neurologists, neuro-oncologists, neurosurgeons, and other members of the multidisciplinary NF1 care team attending the 2021 Child Neurology Society (CNS) Annual Meeting. Designed and developed to provide an interactive overview of novel and emerging data, as well as establish a foundational context of NF1 disease state complexity and intractability, this activity will begin by reviewing revised diagnostic criteria and hallmark aspects of NF1 natural history and clinical presentation in pediatric patients, and describing the genetic etiologies and multi-system pathophysiology that have historically made NF1-associated tumors so difficult to treat. Supported by a dynamic case-based approach intended to encourage audience engagement, attendees will gain insights from key opinion leaders regarding the pivotal shortcomings of traditional NF1 management modalities and the new horizons that have accompanied the emergence of targeted medical therapies, most notably MEK inhibitors. This discussion will be supported by a detailed appraisal of clinical trial data, treatment recommendations, and approved indications for targeted therapies in NF1. Finally, top-level child neurology experts will guide attendees through a case-driven, real-world exploration of the expanding role of the pediatric neurologist as the NF1 paradigm advances into the era of MEK inhibition.

Verena Staedtke, MD, PhD (Activity Chair)
Director of Pediatric Neurofibromatosis
The Johns Hopkins Comprehensive Neurofibromatosis Center
Associate Professor of Neurology
Johns Hopkins University
Baltimore, MD

Michael J. Fisher, MD
The Children's Hospital of Philadelphia
University of Pennsylvania Perelman School of Medicine
Philadelphia, PA

Nicole Ullrich, MD, PhD
Associate Professor of Neurology
Harvard Medical School
Boston, MA

https://www.ceconcepts.com/NF1-CNS

Financial support by Astra Zeneca

VIRTUAL:

Explore the Science for an Approved SMA Treatment and Hear From People Living with Spinal Muscular Atrophy

Thursday, October 21, 3:00 PM EDT

Spinal muscular atrophy (SMA) is a progressive, genetic neuromuscular disease with a broad spectrum of severity in children and adults. Much progress has been made in the understanding of SMA and, today, people living with Type 1, 2, or 3 SMA have options for disease-modifying therapies. In this symposium, a clinician speaker will present the efficacy and safety of an approved SMA treatment and will interview members of the SMA community who have made this treatment part of their SMA management plan. The community speakers will share their journeys from diagnosis onward, including discussions around treatment decisions, assessment of progress, and everyday life with SMA.

Elizabeth Kichula, MD, PhD
Pediatric Neurologist
Attending Physician
Children's Hospital of Philadelphia
Philadelphia, PA

https://genentechrsvp.com/registrations/search?program_code=CM41484

Financial support by Genentech

Session links provided on virtual platform and on CNS website 2021 Annual Meeting page:
https://www.childneurologysociety.org/colleagues/network/cns-annual-meeting/