

Industry-Sponsored Satellite Sessions

ON DEMAND thru January 31, 2022

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.

ON DEMAND:

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

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

- Provide an overview of aromatic L-amino acid decarboxylase (AADC) deficiency and the eladocogene 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 eladocogene 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

ON DEMAND LINK:
<https://www.ptcbio.com/advancing-the-care-of-a-pediatric-neurotransmitter-disorder/>

Financial support by PTC Therapeutics

ON DEMAND:

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

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 $\geq 75\%$ 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

Introduction

Elizabeth A. Thiele, MD, PhD (Program Chair)
Harvard Medical School
Massachusetts General Hospital

Dravet Syndrome: Clinical Updates and Therapeutic Options

Elizabeth A. Thiele, MD, PhD

Seizure Management: From Clinical Trials to Clinical Care

Elaine C. Wirrell, MD, FRCPC
Mayo Clinic

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

Q&A

ON DEMAND LINK:
<https://www.cmeuniversity.com/course/disclaimer/121949>

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