Genetics: Early diagnosis and driving precision medicine in pediatric neurology
Long-term treatment with Brineura® (cerliponase alfa)

Tuesday, February 11, 2020
6:15 – 7:15 AM • Windermere Ballroom X • Hyatt Regency Orlando, FL, USA
Complimentary breakfast will be available for attendees

Welcome & introduction
Angela Schulz, MD, PhD
University Medical Center Hamburg-Eppendorf, Children’s Hospital, Hamburg, Germany

Faculty
Angela Schulz, MD, PhD
Angela Schulz is Pediatrician and head of the research group for Childhood Neurodegenerative Disease at the University Medical Center Hamburg-Eppendorf, Hamburg, Germany. Her main research interests are rare pediatric neurodegenerative diseases, with focus on lysosomal storage diseases.

Raman Sankar, MD, PhD
Raman Sankar is Distinguished Professor of Neurology and Pediatrics at the David Geffen School of Medicine at the University of California, Los Angeles (UCLA), CA, USA, and Chief of Pediatric Neurology at the UCLA Mattel Children’s Hospital, Los Angeles, CA, USA. His main research interests include mechanistic studies on epilepsy and associated comorbidities, and the development of improved diagnostics in the evaluation of pediatric epilepsy. Prof. Sankar is the recipient of the Founders’ Award of the American Epilepsy Society in recognition of his lifetime contributions.

Brineura® (cerliponase alfa) injection for intraventricular use is indicated to slow the loss of ambulation in symptomatic pediatric patients 3 years of age and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2). Brineura is contraindicated in patients with any sign or symptom of acute, unresolved localized infection on or around the device insertion site (e.g., cellulitis or abscess), or suspected or confirmed CNS infection (e.g., cloudy CSF or positive CSF gram stain, or meningitis), any acute intraventricular access device-related complications (e.g., leakage, extravasation of fluid, or device failure), and with ventriculoperitoneal shunts.

Use in Specific Populations
• Safety and effectiveness in pediatric patients below 3 years of age have not been established.
• Brineura has not been studied in pregnancy or lactation.

Contraindications
• Brineura is contraindicated in patients with any sign or symptom of acute, unresolved localized infection on or around the device insertion site (e.g., cellulitis or abscess), or suspected or confirmed CNS infection (e.g., cloudy CSF or positive CSF gram stain, or meningitis).

Meningitis and Other Intraventricular Access Device-Related Infections:
• In case of intraventricular access device complications, discontinue the Brineura infusion and refer to the manufacturer’s labeling for further instructions.

Cardiovascular Adverse Reactions:
• Monitor vital signs before, during, and post-infusion. Monitor ECG evaluations every 6 months.

Vomiting, Seizures, Hypersensitivity, Increased CSF Protein, Hematoma, Headache, Irritability, Pleocytosis:
• Inform caregivers of the signs and symptoms of anaphylaxis, hypotension, bradycardia, and device-related complications.

Long-term CLN2 disease outcomes - Importance of early intervention
Angela Schulz, MD, PhD
University Medical Center Hamburg-Eppendorf, Children’s Hospital, Hamburg, Germany

Q&A
Faculty & audience

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