9:15 am – 10:15 am CDT

**Clinically Meaningful Assessments for Neuromuscular Disorders - Focus on Pompe Disease**

This program will (1) Educate healthcare providers regarding how to recognize the clinical features of Pompe Disease and monitor the disease with clinically relevant parameters; and (2) Highlight the importance of a multidisciplinary approach to managing Pompe Disease.

**Speaker:** Matthew Wicklund, MD

**Sponsored By:** Sanofi Genzyme

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12:00 pm – 1:00 pm CDT

**Expanding Possibilities in the Treatment of Spinal Muscular Atrophy**

Spinal muscular atrophy (SMA) is an inherited, degenerative neuromuscular disease that can result in severe muscle weakness and loss of function over time. Disease severity and rate of functional decline vary, ranging from hypotonia, failure to reach motor milestones, and potentially fatal respiratory complications in infantile-onset SMA, to loss of ambulation and slow but progressive motor function decline in children and adults with later-onset disease. In this session, participants will be introduced to efficacy and safety data from an approved SMA treatment option, including case studies reflecting the real-world spectrum of patients with SMA.

**Speaker:** Perry Shieh MD, PhD

- Health Sciences Associate Professor
- Department of Neurology
- David Geffen School of Medicine at UCLA
- Ronald Reagan UCLA Medical Center

**Sponsored By:** Genentech
Are your patients with hereditary ATTR amyloidosis also suffering from polyneuropathy? Our expert speakers will discuss the importance of recognizing the disease early and how a treatment option for the polyneuropathy of hereditary ATTR amyloidosis affects neuropathy progression and patient quality of life. We look forward to your participation!

Speakers:  
Dr. Said Beydoun, University of Southern California  
Dr. Andrew Darlington, Piedmont Atlanta Hospital  
Jean, Patient

Sponsored By:  
Akcea Therapeutics
Industry Forums
Friday, October 9

9:15 am – 10:15 am CDT
Healthcare professionals and their patients: How can we better manage uncontrolled generalized myasthenia gravis together?
Uncontrolled generalized myasthenia gravis (gMG) means living with unpredictability. In many patients with gMG, the disease course remains uncontrolled and unpredictable. Fluctuating symptoms and the risk of exacerbations can disrupt the daily lives of patients. Effective communication between healthcare professionals (HCPs) and patients is key in evaluating the impact of gMG on patients’ day-to-day lives. We invite you to join us for this virtual symposium where we will hear from our esteemed faculty and an individual living with gMG to discover the importance of holistic patient assessment and enhanced HCP–patient communication in ensuring that the day-to-day impact of gMG is fully appreciated and considered as part of disease management strategies.

Speakers: James F. Howard, MD
Nicholas J. Silvestri, MD
Kelly Davio, Patient

Sponsored By: UCB

12:00 pm – 1:00 pm CDT
ONPATTRO® (patisiran) in Practice
The faculty in this program will share their experiences diagnosing and treating adult patients with the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis. Faculty will review hATTR amyloidosis and the red-flag symptoms of the disease, discuss challenges associated with diagnosing the disease, present two patient case studies, and share the clinical profile of ONPATTRO® (patisiran). For additional information about ONPATTRO, please see the Important Safety Information at www.onpattrohcp.com/important-safety-information and the full Prescribing Information at https://alnylam.com/onpattro-us-prescribing-information.

Speakers: Francy Y. Shu, MD, UCLA Medical Center
John David Eatman, MD, North Kansas City Hospital

Sponsored By: Alnylam Pharmaceuticals
Industry Forum
Saturday, October 10

9:15 am – 10:15 am CDT

Antisense Oligonucleotide Targeted Therapies in SMA and ALS

This industry forum aims to provide an overview of the antisense oligonucleotide (ASO) technology as well as their clinical applicability in the context of SMA and ALS. Dr. Krainer will discuss the science underlying ASO technology, and Dr. Day will share an overview of clinical data collected on nusinersen for SMA and investigational tofersen for SOD1-ALS.

Speakers: Adrian Krainer, PhD
           John Day, MD, PhD

Sponsored By: Biogen