ALS Drug Development: Focus on Fast Skeletal Muscle Activation as a Target for ALS

Jeremy M. Shefner, MD., PhD., FAAN
Kemper and Ethel Marley Professor and Chair of Neurology
Senior Vice President, Barrow Neurological Institute
Chief Medical Officer of Clinical Research, Barrow and St. Joseph’s Hospital and Medical Center
Executive Chair of Neurology, University of Arizona in Phoenix
Chair of Neurology, Creighton University School of Medicine in Phoenix

Friday, November 15, 2019
3:05 p.m., SCOB 228

Abstract
Rational drug development requires an understanding of appropriate targets for both disease modification and functional benefit. Many neurodegenerative diseases are understood incompletely, so that target identification is at best speculative. In this seminar, I will highlight one approach to improve function in patients with amyotrophic lateral sclerosis. The underlying hypothesis is that increasing the force of submaximal muscle contraction can be helpful in a disease characterized by progressive weakness due to motor neuron loss. Starting from in vitro experiments and progressing through late phase clinical investigation, the drug development process will be discussed, and decisions to either proceed or abandon further development will be highlighted. The overall goal of the session will be to improve the attendees understanding of how drugs are developed for neurodegenerative disease in particular and for incompletely treated diseases more generally.

Bio-Sketch
Jeremy Shefner, M.D., Ph.D., is a Board-certified Neurologist specializing in ALS and Neuromuscular Disorders. Dr. Shefner’s research focuses on biomarker development and the clinical therapeutics of amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy. He co-founded the Northeast ALS Clinical Trials Consortium (NEALS), the largest and most active consortium in the world dedicated to ALS. He continues to direct the NEALS outcomes and clinical monitoring cores and is currently the principal investigator for two multicenter clinical trials, as well as a biomarker study evaluating the effectiveness of electrical impedance myography in measuring ALS disease progression.

Dr. Shefner has published approximately 200 papers in peer-reviewed journals and has served on multiple grant review panels. He has also participated in committees organized by the Institute of Medicine to investigate the relationship between military service and ALS, as well as the health effects of Agent Orange on Vietnam War veterans. In 2014, Dr. Shefner received the Sheila Essey Award for ALS Research, presented annually by the American Academy of Neurology and the ALS Association.

Dr. Shefner received his Ph.D. in sensory physiology from the University of Illinois in Urbana-Champaign, and his MD from Northwestern University Medical School in Chicago. He completed his residency training at the Harvard Longwood Neurology Training Program in Boston and completed a fellowship in neuromuscular disease at the Brigham and Women’s Hospital in Boston.