

News Release

American Neuromuscular Foundation
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FOR IMMEDIATE RELEASE

Jerry R. Mendell, MD, Receives 2023 Best Abstract Award for DMD Gene Therapy Research

Rochester, Minn. (July 26, 2023)- The American Neuromuscular Foundation, is excited to announce Jerry R. Mendell, MD, as the recipient of the 2023 Best Abstract Award, for his abstract titled, "Long-Term Safety and Efficacy in Patients With Duchenne Muscular Dystrophy 4 Years Post-Treatment With Delandistrogene Moxeparvovec in a Phase 1/2a Study."

Dr. Mendell is an attending neurologist at Nationwide Children's Hospital, the Dwight E. Peters and Juanita R. Curran Endowed Chair in Pediatric Research at the Abigail Wexner Research Institute, and professor of pediatrics and neurology at The Ohio State University.

Dr. Mendell is a leading authority in neuromuscular (NM) disease research, and his groundbreaking research focuses on the development of gene therapy for Duchenne muscular dystrophy (DMD). His winning abstract reports the outcomes of a long-term, open-label, Phase 1/2a study (Study 101; SRP-9001-101; NCT03375164) evaluating the safety and functional outcomes 4 years post-treatment with delandistrogene moxeparvovec (SRP-9001), an investigational rAAV vector-based gene therapy designed to compensate for missing dystrophin in DMD by delivering a transgene encoding SRP-9001 dystrophin. Dr. Mendell concluded that delandistrogene moxeparvovec was well tolerated 4 years post-treatment.

This study helped support the agent's biologics license application, and in June 2023, SRP-9001 was granted accelerated approval by the FDA for individuals with DMD ages 4-5. Dr. Mendell said, "This accelerated approval is extremely important and really advances the field. It's the first time we'll have systemic gene therapy in the field for DMD."

Dr. Mendell will be recognized at the 2023 AANEM Annual Meeting, Nov. 1-4 at the JW Marriott Desert Ridge in Phoenix, Arizona.

About American Neuromuscular Foundation (ANF)

Based in Rochester, MN, ANF is a nonprofit organization dedicated to strengthening the global effort to cure neuromuscular disease. As part of its mission to promote the advancement of scientific research and education, the ANF offers annual development grants and abstract and fellowship awards to launch research into muscle and nerve disorders by experts in the field. For more information about ANF, visit [neuromuscularfoundation.org](https://www.neuromuscularfoundation.org).

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