Jerry R. Mendell, MD, is Emeritus Professor at Nationwide Children's Hospital where he held the Dwight E. Peters and Juanita R. Curran Endowed Chair in Pediatric Research at the Abigail Wexner Research Institute at Nationwide Children's Hospital. He has been elected to the National Academy of Medicine and recently received the ASGCT Translational Medicine Award that was named in his honor and has been given yearly to accomplished scientists.

He did Neurology residency at Columbia University's New York Neurological Institute. His post-doctoral fellowship at the Medical Neurology Branch of NIH began his career in neuromuscular disease. He has published >400 articles with a focus on neuromuscular disease and authored books on Skeletal Muscle Disease, Peripheral Nerve Disorders, and Gene Therapy.

Early work in DMD described a vascular pathway responsible for muscle damage in Duchenne muscular dystrophy (DMD) now confirmed by the link to nNOS binding sites of muscle. The first breakthrough in treatment was described in 1989, as efficacy of corticosteroids in DMD (Mendell JR, et al. N Engl J Med 320:1592-1597, 1989). Prednisone or one its corticosteroid variants now standard of care for DMD. Since then, research has moved toward molecular-based strategies. In 1999 Dr. Mendell performed the first in-human clinical trial using AAV for gene transfer to skeletal muscle. In March 2007, Dr. Mendell's gene therapy in limb-girdle muscular dystrophy type 2D, demonstrated sustained gene expression for more than 6 months, an important milestone for the field (Mendell JR Ann Neurol 2009;66:290-297). In a similar gene therapy approach for DMD, he demonstrated that expressing the transgene into deleted domain resulted in rejection of the gene product because of transgene immunity (Mendell JR et al N Engl J Med;363:1429-37).

He was instrumental in establishing the international incidence of DMD at birth at 1:5000 (Mendell JR, et al Ann Neurol 2012;71:304-313). Clinical Trials led by Dr. Mendell in exon skipping were noteworthy as the first therapeutic agent to show increased dystrophin expression following long-term exon skipping outcomes demonstrating slowing of disease progression (Mendell al. Ann Neurol 2013; 74:637-47; Ann Neurol 2016; 79:257-271). Eteplirsen (Exondys 51) is approved by the FDA for commercial use.

He was the principal investigator for SMA gene therapy, the first systemically delivered gene showing achieving safety and efficacy (Mendell JR, et al N Engl J Med Nov 2017). This was a major milestone saving the lives of infants with gene delivery by intravenous administration. This work received Science Magazine 2017 Breakthrough of the Year Award. SMA gene therapy has now been approved by the FDA for clinical therapy as Onasemnogene Abeparvovec (Zolgensma®, Novartis, Inc). Based on SMA gene therapy, newborn screening is now established in 49 states throughout US.

Currently Dr. Mendell is actively engaged in systemic delivery of micro-dystrophin-DMD gene therapy. He is the Principal Investigator and as a result gene therapy has been approved by FDA treatment of DMD patients 4-5 years old (Elevidys®, Sarepta, Inc.). Studies are underway to obtain gene therapy treatment for all DMD patients.

Dr. Mendell now serves as a Senior Advisor for Sarepta Therapeutics.