



Editorial Note: Brain disorders & Therapy

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Editorial note on Neuromuscular Disorders

Neuromuscular diseases have a considerable burden on patients, their caregivers, and healthcare systems. These diseases are largely genetic, degenerative, and life-threatening or life-altering. The dystrophy Association (MDA) has been at the forefront of research, therapy development, and early intervention for neuromuscular disease since its foundation in 1950. The MDA Clinical & Scientific Conference, held on March 22–25, 2020, in Orlando, Florida, will mark the 70th anniversary of the MDA. Huge progress has been made in recent years, but the sector has reached a crossroads—strong advocacy programmes from the MDA and other stakeholders are going to be essential to continue the success stories of neuromuscular research and to make sure the readiness of systems to translate these breakthroughs to clinical practice.

MDA is that the largest non-governmental funder of neuromuscular research within the USA, supporting 252 research projects worldwide in 2019, with funding commitment of quite US \$66 million. Since its inception, MDA has invested quite \$1 billion in neuromuscular disease research, funding projects throughout the drug development spectrum, from the earliest discovery stages to clinical trials. MDA-funded breakthroughs include drugs for amyotrophic lateral sclerosis (riluzole), Duchenne dystrophy (eteplirsen, deflazacort, and golodirsen), periodic paralysis (dichlorphenamide), Pompe disease (alglucosidase alfa), and spinal muscular atrophy (nusinersen). Of note, in July, 2007, MDA invested nearly \$750000 in awards to Adrian Krainer and his colleagues at the Cold Spring Harbor Laboratory in NY, for early-stage development of nusinersen, in collaboration with drug developers within the pharmaceutical industry. In December 2016, the US Food and Drug Administration (FDA) approved nusinersen, the primary treatment ever for youngsters with spinal muscular atrophy, the leading genetic explanation for death in infants. For the interpretation of breakthroughs to clinical practice, other elements beyond research investment are crucial – accelerated development of successful therapies; improved trial design informed by current standards of care; efficient patient identification, enrolment, and retention; and

access to therapies for patients. The MDA's Care Centre Network is that the largest network of neuromuscular clinics in the USA, with Care Centres providing multidisciplinary specialist care at over 150 of the nation's top medical institutions, located in 47 US states also as Puerto Rico. Care Centres can provide diagnostic services, deliver therapy, connect patients with clinical trials, and conduct those trials, during a setting designed to supply support to both patients and their caregivers.

In 2018, MDA launched the MOVR (neuromuscular Observational Research) Data Hub, a completely unique data source that will be the foremost comprehensive nationwide collection of real-world data for neuromuscular disorders. The Data Hub are going to be available to a broad community of healthcare providers, researchers, and industry partners, and the data collected will facilitate understanding of the heterogeneity of neuromuscular disorders, measurement of the impact of real-world therapies, identification of patients for brand spanking new therapy trials, and tracking of the natural history of disease. To date, 40 sites are contracted to participate in MOVR, 20 sites have institutional review board approval to start enrolling patients, and 16 sites are active and have already collected data from almost 3000 patients with neuromuscular disorders. The current available data are getting used for early analysis, like benchmarking of clinical care standards. The Data Hub are often also employed by the Network for trial design and recruitment. for instance, MOVR is being used to optimise inclusion criteria, by adjusting criteria to reflect real-world clinical practice and to spot and prioritise study sites. MDA decide to extend this Network and Data Hub to all or any 150 sites across the USA, and partner with other organisations and international collaborators. MOVR will have collected data from more than 7600 patients by 2021, which will be analysed in comparative-effectiveness studies.

The commitment and dedication of organisations like the MDA and their partners has been essential to the reach date and can continue to be integral to reworking the lives of individuals with neuromuscular disorders. Hopefully, MDA can extend its collaborations globally to further accelerate this progress.

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