The field of neuromuscular disease (NMD) research is experiencing tremendous growth as a result of new insights gained through both diagnostic and therapeutic advancements. However, there continues to be a critical data shortage, as data regarding disease progression have not been captured systematically across NMDs nor in centralized, accessible databases. The neuromuscular Observational Research Data Hub (MOVR) was created by the Muscular Dystrophy Association (MDA) to accelerate data collection, maximize drug development, and enhance the impact of new therapies. MOVR is powered by the MDA Care Center network, which consists of over 150 multidisciplinary care centers across the United States. Currently, 60 care centers have activated the MOVR Study Protocol and data from 3,946 participants and 12,376 encounters have been captured. Preliminary analyses of MOVR Data have been performed to characterize the use of approved therapies by participants living with spinal muscular atrophy (SMA), amyotrophic lateral sclerosis (ALS), or Duchenne muscular dystrophy (DMD). For participants living with SMA, the method of diagnosis (i.e., newborn screening, symptom onset) influences the type of therapy that is used and the age at which therapy is initiated. For participants living with ALS, over 75 percent of participants use an FDA-approved therapy, and these therapies are initiated on average less than 6 months after diagnosis. For participants living with DMD, the use of exon-skipping therapy is relatively limited among those participants who have mutations amenable to these therapies, with only 38 percent of participants receiving therapy. Future analyses will be performed to understand how these therapies may be impacting other core data elements captured by MOVR, including medication usage, multidisciplinary referral types, and functional measures. As the first data hub to aggregate clinical and genetic data across multiple NMDs, MOVR continues to demonstrate its ability to transform the NMD space by serving as a powerful tool for clinical trial matching, clinical trial design and feasibility, and as a source for real-world data for pre- and post-approval submissions.

### Approved Therapy Use in DMD Participants

#### Demographics of MOVR Participants with DMD

- **99.6%** of DMD participants are male
- **15.2 ± 6.7 years** average age of active DMD participants
- **5.2 ± 3.6 years** average age at diagnosis
- **93.1%** of DMD participants have a genetic diagnosis

#### DMD Therapeutic Approval Timeline

- **2016**: Exondys 51 approval
- **2019**: Evrysdi approval
- **2021**: Salveo approval

#### Therapy Use in MOVR Participants with DMD

- **94.3%** of DMD participants with exon 5 skipping

### Approved Therapy Use in ALS Participants

#### Demographics of MOVR Participants with ALS

- **56.9%** of ALS participants are male
- **64.5 ± 10.9 years** average age of active ALS participants
- **1.5 ± 1.3 years** average time from symptom onset to diagnosis
- **72.5%** of ALS participants had non-Bulbar onset

#### ALS Therapeutic Approval Timeline

- **1995**: Riluzole approval
- **2011**: Nusinersen approval
- **2017**: Radic劲se approval
- **2018**: Tigrilux approval
- **2019**: Exsensur approval

#### Therapy Use in MOVR Participants with ALS

- **77.8%** of ALS participants have used an FDA-approved therapy
- **5.2 ± 5.6 months** average time from diagnosis to start of an FDA-approved therapy

#### Regional Differences in FDA-approved therapy use are not present

### MOVR as a Centralized Data Hub

About 10 years ago, MDA recognized that there was a significant data shortage in the NMD space and started crafting strategic approaches to accelerate data collection and its use by researchers, clinicians, and drug developers. One strategy that was identified was to leverage the MDA Care Center Network as a source for efficiently capturing clinical data and growing a longitudinal dataset. Each year, over 80,000 medical visits are conducted and over 60,000 individuals living with NMDs receive expert care at these centers.

Data elements captured by MOVR are functional and disease-specific outcome measures that have been identified by KOLs as important to understanding disease mechanisms, tracking disease progression, and implementing standards of care.

### Conclusion

The MOVR Data Hub is experiencing tremendous growth in the number of participants enrolled in this observational study as well as in the number of longitudinal clinical encounters. Standardization of data elements across the 7 indications provides MOVR with the ability to examine the same outcome measures across time. For these analyses, we examined the use of FDA-approved therapies in MOVR Participants living with DMD, SMA, and ALS. The majority of ALS and SMA participants are receiving (or have received) an FDA-approved therapy but relatively few DMD participants have received a therapy. Building these two cohorts (therapy-naive vs. therapy-experienced) provides an opportunity to explore how therapies impact disease progression as well as standard of care practices. The MOVR Data Hub is a valuable tool for monitoring longitudinal response to therapies and could serve as an excellent source for real-world efficacy data for future regulatory submissions.

### Contact the MOVR Team

For access to MOVR Data, please email NDLDMOVR@mdausa.org

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