February 28, 2022

Division of Dockets Management (HFA-305)
U.S. Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: FDA-2021-D-1146: Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products - Guidance for Industry

Dear Sir or Madam,

In service of the neuromuscular disease (NMD) patient community, the Muscular Dystrophy Association (MDA) thanks the Food and Drug Administration (FDA or "Agency") for the opportunity to comment on the Agency's Guidance entitled, "Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products - Guidance for Industry". We are grateful for the Agency's efforts to guide the stakeholder community on how best to utilize registries to support regulatory decision-making.

MDA is the nation's leading nonprofit organization dedicated to transforming the lives of individuals living with neuromuscular diseases through innovations in science and innovations in care. MDA fulfills its mission by funding biomedical research, providing access to expert clinical care and support through its national MDA Care Center Network, and by championing public policies and programs that benefit those we serve. Since inception, MDA has invested more than $1 billion in research grants to accelerate treatments and cures for neuromuscular disorders, making MDA the largest source of neuromuscular disease research funding in the U.S. outside of the federal government.

To support the clinical, research, and drug development efforts ongoing within neuromuscular diseases, MDA launched the neuroMuscular ObserVational Research Data Hub (MOVR). In our comments below, we share how MOVR meets the current guidelines presented in the guidance as well as our plan to meet those guidelines that we do not yet meet. We also ask for clarifications on certain guidelines presented in this document and provide our recommendations for improving this guidance based on our experience with growing and managing MOVR.

MDA's neuroMuscular ObserVational Research Data Hub (MOVR)

About ten years ago, MDA recognized that there was a significant data shortage in the neuromuscular disease 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, which is comprised of over 150 care centers and 2,400 clinical providers across the United States, as a source for efficiently capturing

clinical data and growing a longitudinal dataset. Specifically, each year, over 90,000 medical visits are conducted and over 60,000 individuals living with a neuromuscular disease receive expert care at these centers. Capturing such a dataset would provide valuable knowledge on disease progression for drug development as well as for RWD and RWE in regulatory submissions and post-approval processes. This network also serves as a hub of neuromuscular research activity with over 20,000 individuals participating in clinical trials and natural history studies.

The US Neuromuscular Disease Registry (USNDR) served as MDA's pilot registry. The USNDR actively collected clinic-entered data across four diseases (amyotrophic lateral sclerosis [ALS], Becker muscular dystrophy [BMD], Duchenne muscular dystrophy [DMD], and spinal muscular atrophy [SMA], at 26 care centers from 2013 to 2018. The success of USNDR, including collecting data from approximately 2,700 participants and using these data in an EU regulatory submission, inspired MDA to partner with IQVIA, a leader in human data science technology, to create MOVR. The USNDR dataset was directly transferred into MOVR, and three new diseases were added: Facioscapulohumeral muscular dystrophy (FSHD), Limb-girdle muscular dystrophy (LGMD), and Pompe disease.

MOVR represents the first data hub that will aggregate clinical and genetic across multiple neuromuscular diseases. The core data elements captured across all diseases, include:

- Demographics – disease type, enrollment date, gender, DOB, race, ethnicity, insurance, education, and employment
- Diagnosis – date and age at diagnosis, clinical diagnosis, muscle biopsy, body regions first affected, family history, molecular and DNA results, and gross and developmental motor milestones
- Encounter – encounter date, height and weight, clinical trial participation, surgical history, falls and hospitalizations, medications, mobility, assistive devices, disease progression, spinal conditions and neuroimaging, nutritional and GI therapies, pulmonary and cardiology care, and multidisciplinary care
- Discontinuation – date of withdrawal, reason for study withdrawal, date of death, and cause of death

MOVR data are entered by clinical research staff from the information available in participants' medical records. Data are entered from the initial study enrollment visit through follow-up visits until the participant withdraws from the study, is lost to follow-up, or becomes deceased. The Encounter data is captured at each visit and is the foundation of the longitudinal dataset that could serve as RWD and RWE.

MOVR's Current Data Landscape

As of December 2021, 50 care centers are actively enrolling participants. These sites are classified as adult only, pediatric only, adult and pediatric, and ALS only care centers. The total number of MOVR participants across all sites is 4,222. Of these participants, 1,726 were enrolled directly into MOVR since 2019 while 2,496 participants consented to have their data migrated from the USNDR. A total of 894 participants are no longer actively participating in MOVR. Most of these participants were living with ALS who became deceased (n = 708) while others withdrew consent (n = 69) or were lost to follow-up (n = 72). The average number of encounters

per participant ranges from 1.57 (FSHD) to 3.24 encounters (DMD) while the average number of months between the first and most recent encounter ranges from 11.51 (FSHD) to 26.53 months (DMD). Almost 90% of all electronic case report forms (eCRFs) were marked complete, meaning all required data fields were filled for these forms. These data represent the start of a potential dataset for RWD and RWE used in regulatory submissions.

MOVR's Compliance with FDA's Draft Guidance on Using Registries to Support Regulatory Decision-Making

The below table demonstrates how MOVR satisfies the individual guidelines presented in the FDA Guidance. MDA is open to sharing the documents and policies discussed in the column titled "How MOVR Satisfies Guideline".

FDA Guideline	Satisfied by MOVR?	How MOVR Satisfies Guideline
1. Does the registry have an established data dictionary?	Yes	MOVR provides an updated Data Dictionary following each platform update. It is delivered as an Excel file with individual worksheets for each eCRF, including the seven Diagnosis and Encounter eCRFs.
a. Is it made available for those who intend to use the registry data?	Yes	The data dictionary is made available to those who are interested in learning more about the MOVR Dataset.
b. Does it include data elements and how the data elements are defined?	Yes	Each worksheet in the Excel file contains all of the data elements and definitions from each eCRF, including the seven Diagnosis and Encounter eCRFs for each indication.
c. Does it include ranges and allowable values for the data elements?	No	The dictionary does not currently include allowable values or ranges in its current state. However, the electronic Case Report Form Completion Guidelines (eCCG) provides this information and can be made available to interested parties.
d. Does it reference to the source data for the data elements?	Yes	The Data Dictionary is provided alongside the individual eCRFs to provide a more complete understanding of the data captured by MOVR.
2. Does the registry have rules for the validation of queries and edit checks of registry data?	Yes	MOVR has several rules for validation of queries and edit checks, including: **Data Entry Requirements:**

		• Required Fields: The eCRF cannot be marked as 'complete' until core demographic, encounter, and functional outcome variables are completed by the site (27 fields are required to save as complete). • Automated Edit Checks: A significant number of data entry validations (for example, there are 308 error validations and warnings for DMD) are built into the Electronic Data Capture (EDC) system to prevent errors at the point of entry and facilitate data completeness. **Data Management Program:** • Manual Queries / Listing Review: Manual queries are issued by the MOVR Data Management team based on critical data elements defined in the EDC Data Cleaning Plan.
a. Is it made available for those who intend to use the registry data?	Yes	MDA provides all information regarding the MOVR Platform, EDC, and data management plan when requested by interested parties.
3. Does the registry have defined process and procedure for data collection?	Yes	MOVR data are captured through eCRFs and electronic health record (EHR) integration on a web-based portal. All MOVR data are entered by clinic study staff from the information available in the participants' medical records. Data are entered from the initial study enrollment visit through follow-up visits until the participant withdraws from the study, is lost to follow-up or becomes deceased. This data entry is guided by the eCCG. Extensive site training and quarterly calls with each site are completed to

		answer questions and maintain data integrity.
4. Does the registry have defined process and procedure for data curation?	Yes	There are defined processes, rules, and relevant documentation related to the curation of the data.
5. Does the registry have defined process and procedure for data management?	Yes	The EDC system contains data validations across all capture forms and disease indications to reduce the entry of erroneous or implausible values. For some data fields, previous values are highlighted to alert data-entry personnel to potential errors. Key data fields are required so that a minimum amount of information is collected for a given form. Users at MOVR sites are only able to edit records that were entered by their site. In addition to these automated data-entry checks, the MOVR database is regularly reviewed by the MOVR data management team using the predetermined critical data elements in the Data Cleaning Plan to examine more complex discrepancies and/or errors in the data. This review may result in manual queries issued in the EDC system to sites that require follow-up and potential corrections made to the data.
6. Does the registry have defined process and procedure for data storage?	Yes	The data system and technology architecture are managed by IQVIA resources and hosted in a secure cloud. Reporting, logging, and system management are hosted in IQVIA's datacenter. IQVIA is accountable for all hardware and software management, and SLAs. IQVIA conducts backup procedures of all data daily and replicates all production backups to another geographic region in the US. IQVIA retains daily and weekly backups for a period that is sufficient for operational recovery.

7. Does the registry have defined process and procedure to ensure that data within the registry can be confirmed by source data?	No	A source document verification plan is under development now.
8. Does the registry have a plan for how patients will access and interact with the registry data and the registry's data collection systems?	No	MOVR does not currently allow participants to view or interact with their data. Further discussion on this topic is in below sections.
9. Does the registry have a plan for how researchers will access and interact with the registry data and the registry's data collection systems?	Yes	The MOVR Data Governance Policy covers the following areas: • An overview of MDA's Roles and Responsibilities • Authorized and non-Authorized Data Use • Data Ownership • Publication Rights • Fees, if applicable. Researchers must review and sign this policy before a data request form can be submitted. Data requests follow a formal review process to ensure that there is scientific merit, that information requested is available within MOVR, and that the proposed project design and analyses will produce meaningful scientific findings. Requestors must follow certain requirements to maintain security and privacy of participants. MDA is ultimately responsible for maintaining the privacy and security of participants' data in compliance with, but not limited to, HIPAA and the Health Information Technology for Economic and Clinical Health (HITECH) Act. Therefore, MDA sets the standard measures that must be met by all Requestors. The MOVR Research Advisory Committee (RAC) reviews any requests that fall outside of the authorized and non-authorized uses

		described in the MOVR Data Governance Policy. All regulatory and legal requirements must be met before data are securely delivered. Data is delivered as a Data Download Report or transformed into CDISC if preferred by the data requestor. Further, MOVR is currently working to provide external researchers with the ability to interact with the data on a custom visualization and reporting platform created specifically for MOVR Sites. Requestors are required to submit an annual summary report on the progress of the project and outcomes. Non-participant researchers do not have access to the MOVR Platform, where data entry occurs. Researchers only have access to de-identified aggregate data or results from analyses conducted by MDA or its partner IQVIA.
10. Does the registry have a plan for how clinicians will access and interact with the registry data and the registry's data collection systems?	Yes	For clinicians who are not affiliated with a MOVR Site, the same processes described for Question 9 must be followed to access and interact with MOVR Dataset. However, for clinicians who are affiliated with a MOVR Site, a custom visualization and reporting platform is available for them to view and analyze data collected by their site. To view and interact with the MOVR de-identified aggregate dataset, the policies and procedures detailed above for researchers must be completed. Access to the MOVR Platform is only available to the principal investigator of

		the MOVR Site and only their site information can be viewed.
11. Does the registry have terms and conditions for use of the registry data by parties other than the registry creator?	Yes	Similar to researchers and clinicians from Questions 9 and 10, all data requests are formally reviewed, and the same policies and procedures must be followed before data is made available.
12. Does the registry conform with 21 CFR part 11, as applicable, including maintenance of access controls and audit trails to demonstrate provenance of the registry data and support traceability of the data?	Yes	MOVR data is collected, managed, and hosted on IQVIA's Registry Platform (IRP). IRP holds high standards in managing and maintaining the System's Information Technology architecture in alignment with both the Health Insurance Portability and Accountability Act of 1996 and its implementing rules (HIPAA) and 21 CFR part 11, to the extent applicable. IRP undergoes an annual independent HIPAA Risk Assessment and the Data Center where IRP is hosted undergoes an annual ISO 27001 assessment.
13. Does the registry adhere to applicable jurisdictional human subject protection requirements, including protecting the privacy of patient health information?	Yes	MOVR data is collected under participant consent and with IRB approval of the MOVR research protocol (both central and institutional where appropriate). Appropriate use of the data for research purposes is managed by the MOVR Data Governance Policy, and contracts with researchers, all of which is overseen by the MOVR RAC. The IRP implements administrative, physical, and technical safeguards to protect the availability, confidentiality, and integrity of Protected Health Information (PHI) and any other confidential data entered into the system. All such safeguards are in accordance with applicable federal and state laws. IRP follows industry leading SOPs and guidelines in the following areas to

		ensure confidentiality and security of information: • Confidentiality • Data Ownership • Advanced Endpoint Protection • Subcontracting / Outsourcing • Security and Training • Encryption and Data Transmission • Audit Logs • Physical and Environmental Security • Incidence Response and Reporting Only authorized site personnel have access to the fully identified information for participants at their site. Personnel from one site are not able to view identifiable data about participants enrolled at another site. For aggregate data, in order to ensure the highest utility of the data while protecting the privacy of MOVR participants, a de-identification solution was identified in partnership with Privacy Analytics, an IQVIA company. To de-identify the MOVR dataset, a Re-identification Risk Determination (RRD) is conducted to review and assess the re-identification risk of the dataset. De-identification standards used for this evaluation are consistent with the HIPAA Privacy Rule's Expert Determination standard. The determination stipulates changes that are required to reduce the re-identification risk, which are then implemented to create a non-identifiable dataset.

14. Did the registry consult with an institutional review board or independent ethics committee when developing the registry to review data collection and other procedures associated with the registry?	Yes	Each MOVR site obtains institutional review board approval of the study protocol and written informed consent and assent, as appropriate, from each MOVR participant and/or their legal guardians. As allowed in the study protocol, some sites have implemented remote or e-consenting procedures. This flexibility has been essential during the COVID-19 pandemic, as enrollment and data entry remain a priority despite fewer in-person visits. MOVR RAC advises MOVR on medical and scientific matters, provides feedback and guidance on data use and publication policies, and reviews data requests that fall outside of authorized and non-authorized uses of data from academic and life sciences research collaborations.
15. Do the registry personnel and processes in place during data collection and analysis provide adequate assurance that errors are minimized, and that data integrity is sufficient?	Yes	MDA Care Centers are specialized, multi-disciplinary neuromuscular clinics, funded by MDA to conduct visits on a regular (i.e., weekly, monthly) basis. MDA's MOVR team works collaboratively with Care Centers to evaluate the feasibility of their institution participating as a MOVR Site based on defined selection criteria, such as patient volume across the seven disease indications collected by MOVR and overall clinical research involvement. Participating sites are provided with extensive training to support site activation. Regular training sessions are offered for new study staff at the site, and quarterly calls are held with each site to ensure active study participation.

		In line with the data quality procedures outlined in Question 2, Source Data Verification is being implemented in MOVR in 2022.
16. Does the registry have policies and procedures in place for validating the electronic systems used to collect registry data?	Yes	MOVR follows standard operating procedures outlining the computer system validation and software development lifecycle when delivering electronic systems. The SOPs are based on the following: • 21 Code of Federal Regulation Part 11 • Good Documentation Practice • GAMP5 (Good Automated Manufacturing Practices) • SOX (Sarbanes-Oxley) • GDPR (General Data Protection Regulation) • EudraLex Vol 4 Annex 11 Computer Systems • PMDA (The Pharmaceuticals and Medical Devices Agency) • MHRA (Medicines and Healthcare products Regulatory Agency) • ITIL (Information Technology Infrastructure Library) • GCP (Good Clinical Practices) • GLP (Good Laboratory Practices)
17. Are the formats and definitions of the data entered in the registry consistent over time?	Yes	Formats and definitions are consistent. If revisions are made, trainings and documentation is provided and kept up to date to show changes and timestamped.
a. Are changes in diagnostic criteria or clinical definitions accounted for and documented?	Yes	The eCCGs are version controlled and provide details concerning changes in clinical definitions.
18. For an electronic database, does the registry implement and maintain version control by documenting the date, time and	Yes	All data entered into MOVR Platform has an audit trail that tracks the date, time, and originator of the data.

originator of data entered in the registry?		
19. For an electronic database, does the registry perform preventative and/or corrective actions to address changes to the data (including flagging erroneous data without deleting the erroneous data, while inserting the corrected data for subsequent use)?	Yes	The EDC system contains data validations across all capture forms and disease indications to reduce the entry of erroneous or implausible values. For some data fields, previous values are highlighted to alert data-entry personnel to potential errors. Key data fields are required so that a minimum amount of information is collected for a given form.
20. For an electronic database, does the registry ensure data transferred from another data format or system are not altered in the migration process?	Yes	MOVR data can be transformed and hosted in the CDISC SDTM standard, which is one of the required standards for data submissions to the FDA. IQVIA validates the process prior to the implementation to ensure the integrity of the data during the transformation process.
21. For an electronic database, does the registry seek to integrate data in the registry that were previously collected using data formats or technology that are now outdated?	No	Legacy USNDR data was migrated into MOVR prior to its launch. Data validations are consistent across the USNDR and MOVR data sets.
22. For an electronic database, does the registry account for changes in clinical information over time (such as criteria for disease diagnosis)?	Yes	eCRFs have been reviewed and updated by expert clinicians, researchers, and key opinion leaders to satisfy the current diagnostic criteria. Further updates will be conducted on an ad hoc basis at a minimum by the MOVR RAC as we develop a more systematic approach.
23. For an electronic database, does the registry explain auditing rules and methods used and the mitigation strategies used to reduce errors?	No	Now that there are sufficient data in MOVR, MDA's MOVR team and IQVIA are discussing ways to reduce common errors in data entry. Most errors arise in free text fields. Our goal is to integrate the use of more drop-down lists that contain those items that are most often entered as free text.
a. Does it describe the types of errors that were	No	Currently, MOVR does not have an auditing plan for how to keep track of

identified based on audit findings and how the data were corrected?		errors that will need to be corrected but MDA is working to develop these protocols.
24. Does the registry perform routine descriptive statistical analysis to detect the extent of any missing data, inconsistent data, outliers, and losses to follow-up?	No	Currently, MOVR does not perform routine statistical analyses to detect the extent of missing data and outliers. However, the required fields and automated edit checks described in Question 2 help limit these. Losses to follow-up are completed through the Discontinuation eCRF.

As demonstrated in the table above, MOVR satisfies many of the guidelines that would be required for the use of MOVR data in regulatory submissions according to this FDA guidance. For those guidelines that we currently do not satisfy, MDA is working diligently with its data technology vendors, including IQVIA and DNAnexus, to develop strategic approaches that would ensure MOVR's compliance.

Requests for Clarifications

MDA is grateful for the FDA providing this guidance as it allows us to thoroughly review MOVR, including its policies and procedures, data systems and standards, and data use. However, there are two main aspects of the guidance on which we would like further clarification: (1) source data verification and (2) patient access to registry data and platform.

Source Data Verification

Establishing and maintaining data integrity are important aspects of the conversation between MDA and current and potential users of MOVR. MDA currently implements extensive training sessions with clinical research staff at MOVR Sites who are responsible for data entry, and the MOVR Platform harbors multiple electronic data checks at time of data entry to ensure that data captured by MOVR accurately reflects the electronic health record (EHR).

Source data verification (SDV) is a critical component of the regulatory submission process for clinical trials and investigations. MDA believes that SDV also plays a vital role in the success of a registry, especially since a strategic goal for MOVR is to grow disease-specific datasets in a way that would allow them to serve as a natural history study and/or an external control arm comparator. MDA is planning to implement SDV in an annual audit, but we are unable to find certain recommendations and guidance from the Agency on how SDV should be performed across a registry.

This draft guidance recommends that registries have processes and procedures in place for ensuring that errors are minimized and data integrity is sufficient but does not provide details for what these processes and procedures should entail. Since SDV is a requirement for clinical trials, and many registries are building datasets to accompany clinical trial data, MDA requests the

FDA to clarify whether it is expecting that SDV be performed by registry developers prior to any association with a clinical effort.

If SDV is expected, MDA urges the FDA to consider what level of verification is sufficient and feasible. We understand that every registry is different and that SDV could look differently depending on the type of registry. For example, MOVR is actively capturing data from 50 clinics and seven diseases. Would the level of verification necessary be determined by the number of clinics or the number of participants? Would each disease be expected to undergo the same level of SDV or is the registry treated as a single entity and differentiating by disease when conducting SDV is unnecessary? Further, if a registry provides data for a regulatory submission, is the sponsor required to perform its own SDV on the data being submitted in addition to any SDV conducted by the registry owner?

As stated above, MDA is committed to growing and managing datasets that can be used for regulatory submissions. Clarity around SDV for registries would allow MDA to establish and implement a SDV plan for MOVR that is compliant with FDA. An FDA-compliant registry could serve as an incentive for sponsors to use these data in their regulatory submissions, which may reduce the time and financial burdens imposed on the sponsor, the FDA, and other parties involved. Ultimately, reducing these burdens could potentially accelerate the availability of life-changing therapies.

Patient Access to Registry Data and Platform

The Guidance suggests that there should be defined processes and procedures for how patients, researchers, and clinicians will access and interact with the registry data and the registry's data collection systems. Currently, MOVR only captures clinic-entered data. Therefore, only approved clinical research staff at MOVR sites have access to the MOVR Platform (data collection system) for data entry.

This access is further restricted such that clinical research staff can only view and enter data from their respective site; access to data entered by another site is restricted in the MOVR Platform. However, the principal investigator at a MOVR site may request access to the MOVR dataset via a Data Request Form to view the de-identified aggregate MOVR dataset. Unaffiliated researchers do not have access to the MOVR Platform but may request access to the de-identified aggregate MOVR dataset via a Data Request Form. The Data Governance Policy details the acceptable use cases of the MOVR dataset and therefore determines if access is granted. Any requests that fall outside of the Data Governance Policy are reviewed by the MOVR RAC.

MOVR participants (patients) do not have access to their own data captured in MOVR, the MOVR Platform, or the de-identified aggregate MOVR dataset. Should MDA assume that processes and procedures for patient access to data be in place for registries that capture patient-reported outcomes or patient-entered data whereas this requirement may not identically extend to clinic-entered data? Are there specific instances where a patient should have access to the registry data and the data collection systems? Further clarification from the Agency on these questions would be helpful.

Recommendations for the FDA

The rare neuromuscular disease community is experiencing a surge in therapeutic development, including disease-modifying therapies. Nearly 200 products are in the therapeutic pipeline for neuromuscular diseases, with almost half of these products at preclinical versus clinical stages of development. Between 2013 and 2018, the number of products in clinical trials for neuromuscular diseases increased fivefold, from around 20 to 100. In total, over fifteen products are approved by FDA for a rare neuromuscular disease.

MDA is highly aware of the time and financial burdens of the regulatory submission process for the sponsor, the FDA, and any other organization providing data or viewpoints for FDA to consider. Reducing these burdens would greatly benefit all parties. Consequently, we are eager to find ways to lower the time and financial burdens on sponsors when submitting RWE to FDA as part of a regulatory submission.

One such way to reduce this burden is to create a certification or qualification program that registries can complete to demonstrate that they are FDA-compliant and a reputable source for RWD. This qualification program could allow for registries to prove compliance with the recommendations put forward by the Agency in this guidance without having to reassert compliance with every product submission, thus greatly reducing the resources needed for both the sponsor and the FDA

For example, a single registry may provide RWD for multiple applications submitted by different sponsors. Under our current paradigm, the FDA may have to complete the same processes and procedures to verify and validate both the registry and the data being submitted. A qualification program would allow a registry to prepare standardized documents to help sponsors with the submission process and the FDA can be confident in the integrity of the data being submitted. Therefore, upon inclusion of a registry's data in an application, the FDA would see that the registry has already satisfied all requirements and the focus can be on the data included rather than the processes and procedures used to collect, store, and transform the data.

This qualification program can join the existing drug development tool qualification programs as efforts that streamline and reduce the resources needed to use innovative approaches to therapeutic development and regulatory decision-making. Already the animal model qualification program, the biomarker qualification program, and the clinical outcome assessment qualification program are working towards these goals. A registry qualification program could similarly transform therapeutic development efforts, particularly in rare neuromuscular diseases.

In conclusion, MDA created MOVR to improve health outcomes and accelerate drug development. MOVR's foundational goals are to understand the course of disease, increase access to clinical data, speed up clinical trial recruitment, and predict disease progression. However, MDA is committed to growing MOVR as a resource to support study and trial feasibility and design, and as a data hub for post-approval follow-up studies. By leveraging MDA's strong, historical relationships in the medical, scientific, and patient communities, and utilizing the data platform to capture clinical data from visits happening already, MOVR is

poised to overcome the current challenge of industry-wide data shortages in rare neuromuscular diseases with a unique level of stability and scalability.

We are grateful for the opportunity to comment on FDA's efforts to expand the use of real-world data in regulatory decision making. For questions regarding MDA or the above comments, please contact Paul Melmeyer at 202-253-2980 or pmelmeyer@mdausa.org.

Sincerely,

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