MOVR FREQUENTLY ASKED QUESTIONS

Contact: MDAMOVR@mdausa.org

1. What is MOVR?

The MDA MOVR (neuroMuscular ObserVational Research) Data Hub™ is an information platform that aggregates comprehensive clinical and genetic information for individuals seen at MDA Care Centers. MOVR traces its origins to the MDA U.S. Neuromuscular Disease Registry, launched in 2013, which aimed to establish a longitudinal clinical data set to help enhance the understanding of multiple neuromuscular disorders.

2. What are the goals of MOVR?

- **Optimize Health Outcomes**
  - Support the development and refinement of standards of care
  - Provide insights for quality improvement
  - Inform clinical best practices
- **Accelerate Therapy Development**
  - Identify potential participants for clinical trials
  - Identify clinical trial feasibility and design
- **Drive Understanding of Disease**
  - Characterize genotype-phenotype correlation
  - Combine patient perspectives with genetic and clinical data sets
  - Collect insights on pre-symptomatic infants identified via newborn screening
  - Better characterize the patient experience
  - Track natural history of disease
  - Gather and assimilate data gathered outside a clinical trial (also known as real world data-RWD)
  - Demonstrate how therapies are changing the course of disease

3. What diseases are included in MOVR?

People living with any of the seven diseases listed below can participate in MOVR:

- **Amyotrophic Lateral Sclerosis (ALS)**
- **Becker Muscular Dystrophy (BMD)**
- **Duchenne Muscular Dystrophy (DMD)**
- **Facioscapulohumeral Muscular Dystrophy (FSHD)**
  - FSHD1
  - FSHD2
- **Limb-girdle Muscular Dystrophy (LGMD)**

- LGMD1A (MYOT/Myotilin)
- LGMD1B (LMNA/Lamin A/C)
- LGMD1C (CAV3-Caveolin-3)
- LGMD1D (DNAJB6/HSP40)
- LGMD1E (DES/Desmin)
- LGMD1F (TNPO3/Transportin 3)
- LGMD1G (HNRNPDL/Heterogeneous nuclear ribonucleoprotein D-like)
- LGMD1H
- LGMD1I (CAPN3/Calpain-3)
- LGMD2A (CAPN3/Calpain-3)
- LGMD2B (DYSF/Dysferlin)
- LGMD2C (SGCG/Gamma-Sarcoglycan)
- LGMD2D (SGCA/Alpha-Sarcoglycan)
- LGMD2E (SGCB/Beta-Sarcoglycan)
- LGMD2E (SGCD/Delta-Sarcoglycan)
- LGMD2G (TCAP/Telethonin)
- LGMD2H (TRIM32/Tripartite motif containing 32)
- LGMD2I (FKRP/Fukutin-related protien)
- LGMD2J (TTN/Titin)

- **Pompe Disease**
  - IOPD (Infantile Onset Pompe Disease)
  - LOPD (Late Onset Pompe Disease)
- **Spinal Muscular Atrophy (SMA)**
  - SMA Type 0 (symptoms present at birth)
  - SMA Type 1 (symptoms present by 6 months; does not sit unassisted)
  - SMA Type 2 (symptoms present between 7 - 18 months, can sit unassisted but does not stand or walk independently)
  - SMA Type 3 (symptoms present after 18 months, can stand, and walk independently)
  - SMA Type 4 (symptoms present late teens to adulthood)
  - Non-SMN SMA (non-5Q SMA, SMA LED)
  - Other
  - Unknown

4. **With MOVR operational, does this mean the MDA will no longer promote the National ALS Registry?**
MDA is still contracted with the ATSDR/CDC to promote the National ALS Registry. This is an ongoing contract which has been renewed for several more years.

5. Does MOVR have a clinicaltrials.gov identifier?

It is MDA's intention to list MOVR on clinicaltrials.gov. Until we have a reliable plan to obtain site information and support the intake of participant inquiries, we will not do so. As soon as we have this information, we will notify all MOVR sites.

6. Will the list of MOVR sites be located on the MDA website?
The current MOVR site list can be obtained by reaching out to MDAMOVR@mdausa.org.

7. What happens when a patient wants to participate in MOVR but the hospital at which they receive care is not a MOVR site?
There is not an option to enroll patients outside of the designated MOVR site. As the program grows, sites will be added, and this will allow for additional patients to enroll. Patients and families, as well as interested sites, are free to reach out to MDAMOVR@mdausa.org with questions or a list of participating sites.

8. What happens when patients want to be a part of MOVR and do not have a condition that data is currently collected for?
If a patient does not have one of the 7 diseases collected in MOVR, they are not able to participate at this time. As further information is gathered about neuromuscular diseases and as a resources permit, MDA intends to add more disease care report forms (CRFs) to the MOVR database.

9. What qualifies as an eligible MOVR patient and visit?
An eligible MOVR visit is one that takes place within the MDA Care Center network with a patient who is clinically diagnosed with one of the seven diseases listed above. The participant must complete the consenting process to enroll in this study. Since this is strictly an observational study, there are no other inclusion/exclusion criteria as we are only collecting data based on standard of care.

10. What is the digital health module and when will it be available?
The digital health module is not yet developed. Sites will be notified once these launches go live. The digital health module is a way to (electronically) capture patient reported outcomes (ePROs), which demonstrate the ways in which the disease impacts a participant's everyday activities, experiences, and symptoms. MOVR is working to develop and incorporate ePROs through smartphone apps and other technological advancements, allowing patient-driven data to better inform the work clinicians, industry and even MDA does to advance treatments. The digital health module will be a link to these smart technologies allowing patients and families to provide information for a variety of uses including patient satisfaction and Quality of Life (QOL) surveys. Additional information will be made available as the development of the PRO progresses.

11. Will a Central Institutional Review Board (IRB) be used?
All participating sites are encouraged to use Advarra or WIRB as their Central IRB. The use of a central IRB reduces the administrative burden and improves consistency across sites. Advarra and WIRB are part of the SMART IRB system, which most institutions use. There are circumstances where the central IRB cannot be used. In these cases, the Sponsor will allow for use of a site's local IRB as the IRB of record upon review.

12. Are there any IRB approved materials available for use when engaging potential participants?
The participant/family one-pager has been approved by the IRB and is currently available for site use. This one-pager can be requested from the MOVR team at MDAMOVR@mdausa.org.

13. Is the study FDA-regulated?
FDA regulates scientific studies where the goal is to provide evidence of an investigational new drug, biologic and/or medical devices for safety and effectiveness. Since the MOVR registry is observational in nature, it is not FDA-regulated.

14. What are the necessary qualifications of a Principal Investigator (PI) for MOVR?
PI qualifications are as follows:

- Physician
- Current US Medical License
- Absence of professional conduct issues
- Aware of and willing to follow Good Clinical Practices (GCPs) and FDA regulations regarding the conduct of clinical trials.
- Knowledgeable and willing to follow state and local laws regarding medical licensure and medical practice requirements in addition to sponsor requirements.
- Committed to ensuring high quality data collection
- Has access to necessary resources to complete site activation including contracting, IRB submission, training, recruiting and enrolling participants and timely data entry.

There are specific circumstances where someone outside of a physician may serve as an interim Principal Investigator for MOVR, if approved by MOVR leadership. If your institution is facing challenges or going through a transition phase, please reach out to the MOVR team to develop a plan of action. We would be happy to help accommodate and support your team during these times.

15. Is MOVR data different than the regular data collected during a patient's MDA Care Center clinic appointment?
No. MOVR collects data found in your medical record from your MDA Care Center appointment. Since this is strictly an observational registry, this means clinicians are not required to do anything more or less than they already have outlined their treatment plan. Being enrolled in this study will not change the course of your care.

16. How is patient privacy protected?
IQVIA (formerly Quintiles, Inc) is a leading global provider of advanced analytics, technology solutions, and clinical research services to the life sciences industry. IQVIA are the ones who created and host the MOVR platform. IQVIA uses industry technology standards to implement administrative, physical, and technical safeguards to protect the availability, confidentiality, and integrity of Protected Health Information (PHI) and any other confidential data entered in the MOVR platform for a site. All such safeguards are in accordance with applicable federal, international, and states laws. If you'd like further detail around MOVR data security, please email MDAMOVR@mdausa.org.

17. Who can obtain patient consents?
The Principal Investigator (PI) is responsible for overseeing all aspects of the MOVR study at his or her site; therefore, the PI is responsible for delegating the consent process appropriately. Generally speaking, a study coordinator is responsible for the consenting process. Staff members performing the informed consent process should be trained and educated on the study protocol and be clearly listed on the Study Site Training Log and Delegation of Authority Log.

18. Can MDA Care Specialists, Resource Specialists, or other MDA staff consent patients for MOVR?
No. MDA staff are available to speak with patients and families about the MOVR registry and other MDA supports, however, they are not able to consent participants or contact participants unless they are part of the study team, trained, and have consenting tasks delegated to them in the Delegation Log by the Principal Investigator.

19. Do participants who have previously given consent for the prior Neuromuscular Registry (USNDR) need to give additional consent for MOVR?
Yes. The dataset and eCRFs have changed to support the MOVR Data Hub study. All previously consented subjects will need to be re-consented, so they are informed about this new data set.

20. Is non-5Q SMA eligible for enrollment?
Yes, select “Non-SMN/SMA” from the “SMA Classification” dropdown menu in the diagnosis case report form.

21. How does my institution become a MOVR site?
Each prospective MOVR site must complete a site feasibility questionnaire prior to engaging in the site start-up process. After that, execution of a site participation agreement, securing IRB approval and completing site initiation visit (SIV) training is required to participate in MOVR. To learn more, contact MDAMOVR@mdausa.org.

22. Can two clinics within the same institution (i.e., pediatric and adult, or general adult and ALS) be considered one MOVR site?
Yes. The primary consideration is ensuring the PI and any sub-Is are aligned and utilizing adequate support to participate in the registry. A discussion with practice management might be needed to ensure the arrangement is approved. To learn more, contact MDAMOVR@mdausa.org.

23. What happens if a MOVR site has staffing changes after we’ve activated the MOVR protocol?
If you have any kind of staffing changes throughout the course of your study, please notify the MOVR team immediately for next steps (MDAMOVR@mdausa.org). Any changes with the PI/Sub-Investigator will require contacting the central IRB to initiate updates.

24. What happens to data from the Neuromuscular Registry entered since 2013?
MOVR traces its origins to the MDA U.S. Neuromuscular Disease Registry launched in 2013 to better understand how neuromuscular diseases develop and progress and to identify which treatments lead to the best health outcomes. To the extent possible, pre-existing data from your site will be migrated into MOVR before you go live. Due to modifications in the case report forms (CRF) and errors or incompleteness of the data, not all legacy data will be able to be migrated. Data migration will be conducted by IQVIA.

25. Who oversees data entry at the site?
The Principal Investigator is responsible for overseeing all aspects of the MOVR study at his or her site; therefore, the PI is responsible for delegating data entry appropriately at their site. Usually, a study coordinator or data entry specialist is responsible for MOVR data entry. MOVR data entry should only be completed by trained personnel.

26. Who can be a user in the MOVR platform at a site?
Site study staff who have completed MOVR site training and are identified as staff who need access to the data entry platform can be MOVR users. After site training is complete, MDA will approve each user and process the creation of user-specific accounts. Sites will need to work with the MDA MOVR team to create, maintain and remove appropriate user accounts as study staff changes.

27. When will sites receive training on data entry?
After a site has a fully executed Site Participation Agreement and received IRB approval, the MDA MOVR team works with the site staff to schedule a 2-hour (remote) site initiation visit (SIV) training. The MDA MOVR team will conduct a multi-faceted training including webinar presentation, platform demo videos, ISF binder review, and Q&A. Your MDA MOVR team will be available from that point on for any questions, concerns, and training surrounding data entry, if needed.

28. Will guidelines for data entry be provided?
Yes. The “eCRF Completion Guidelines” document was created to walk through each case report form. This is located in the MOVR Library. This Q&A document can also be used as a reference for data entry questions and will be updated as more commonly asked questions arise. Additionally, the MDA MOVR team is available for any data entry questions (MDAMOVR@mdausa.org) as they arise.

29. How long will it take to abstract one participant’s data?
Data abstraction refers to the process of collecting clinical data from the medical record and entering it into another system. There are many factors contributing to data abstraction time including the number of case report forms (CRFs) requiring completion and the site’s electronic medical record system. A new participant (initial visit) requires completion of 3-4 case report forms. MDA and IQVIA have designed these forms with the intent to allow completion within approximately 45-50 minutes. Each additional visit will require one completed case report form. This form should take less than 40 minutes to complete. You can anticipate the time needed to complete these CRFs to decrease as you become more familiar with the MOVR platform.

30. Will MOVR participants be linked to a GUID subject ID number?
Yes. IQVIA has their own internal GUID framework for linking participants across Care Centers and registries within the IRP (IQVIA Registry Platform) system. The GUID framework is configurable to use any set of fields that are available in the database.

31. Who do I contact if I need technical help with the MOVR platform?
If you need any assistance with the MOVR database, please contact the study mailbox (MDAMOVR@mdausa.org). The MOVR team can support you with account creations, deactivations, password resets, troubleshooting, in-line validation messages, completion guidelines and more.

32. How can sites access their data in MOVR?
Each site shall retain ownership of the data it submits to MOVR. Platform logins and temporary passwords are issued to appropriate site staff after completion of training and delegation. Sites can access their data directly through the MOVR data entry platform managed by IQVIA at any time. MOVR is happy to help with data analytics upon request.

33. How can the de-identified aggregate data in MOVR be accessed? Is there a cost?
Access to specific de-identified aggregate data will be granted after review of a request form by the MOVR Data Governance Committee and approval of the request. If the requestor specifically asks for help with data analysis, potential costs are revealed up front prior to moving forward. Requests for data should be sent to MDAMOVR@mdausa.org.

34. Who de-identifies the data?
IQVIA. Sites enter data into the MOVR platform and IQVIA encrypts it when transmitted over the Internet or on any untrusted network using Strong Encryption. The MOVR database is hosted by IQVIA and secured from external access via both policies and technical safeguards.

35. Will this data be shared?
The data will be shared for purposes related to clinical quality improvement, to better understand trends in the population of individuals with neuromuscular conditions, to improve understanding of the causes of illness, to identify the genetics associated with neuromuscular conditions, to learn about disease progression, treatment, outcomes, and survival; to develop educational and other materials for publications; to answer research questions; to provide outcome-related information for the development and improvement of MOVR offerings and services. The specifics around when and how data will be shared are governed by the MOVR Data Governance Policy, which was determined by the MOVR Research Advisory Committee, a formal group comprised of leaders in the neuromuscular disease space.

36. Will data be sold to industry?

Deidentified (coded) data will be provided to industry to support approved research initiatives per the MOVR Data Governance Policy and review of the Research Advisory Committee. If you have questions about this, please reach out to the MOVR team directly.

37. Can sites publish data from their own patients? Can sites publish data from de-identified aggregate data in MOVR?

Sites can publish their own patient data as well as aggregate registry data provided that they submitted the publication to MDA as acknowledgement prior to submission of a manuscript or paper to a third party for publication.

38. Can participants access their own data?

As this registry collects information that is taken directly from a patient's electronic medical record, patients can request access to their medical records through their neuromuscular provider or institution to see what is being entered into MOVR. In the future, as additional data points, such as patient reported outcomes, are included in MOVR, there will be mechanisms by which patients can access the additional information they supply. We will share more details with sites when we are able.

39. Will the data from this study be made available to the public?

Data will be made available to the public via publications reviewed and approved by MOVR and/or provided as part of a regulatory submission for a new drug, biologic or device. This data will always be de-identified.

40. What are MDA's facilities and administrative rate (F&A Rate) policy?

MDA does not pay an F&A rate. If a site would like to cite inclusive F&A rates, they can discuss this with the MOVR team at MDAMOVR@mdausa.org.

41. Will funds be provided to offset the costs of data entry?

MDA understands that significant time is involved in entering data into MOVR. Therefore, MDA will provide funds to help offset the costs of data entry. These funds will be in addition to your current Care Center grant and are set out in detail in Exhibit A of the Site Participation Agreement (page 11).

42. How often are payments issued?

Data entry payments will be calculated and released quarterly based on CRF completion for the preceding quarterly period. The MDA MOVR team manages these payments so there is no need for sites to invoice MDA.

Start-up payments are paid upon site activation. The MDA MOVR team manages these payments so there is no need for sites to invoice MDA.
IRB payments are made upon invoicing by the central IRB or local IRB to MDA.

43. What is IQVIA and what is their role in MOVR?
IQVIA™ – The Human Data Science Company™ -- is dedicated to helping its customers improve health outcomes around the world by mobilizing unparalleled data, technology, expertise, and analytics through services and offerings focused on improving human health. Formed in October 2016 through the merger of Quintiles, (the world's largest contract research organization, founded in 1982), and IMS Health, (the world's largest healthcare data company, founded in 1954), IQVIA's approximately 55,000 employees conduct operations in more than 100 countries.

Powered by the IQVIA CORE™, the company works at the intersection of large-scale analytics, transformative technology, and extensive domain expertise to help clients across a variety of health care sectors tap into a deeper understanding of diseases, human behaviors and scientific advances to advance their path toward cures. Its clients include health care providers and professional associations, biotechnology, medical device, pharmaceutical companies, medical researchers, government agencies, payers, and other healthcare stakeholders.

IQVIA has been supporting USDNR since 2013 and is releasing MOVR on the new IQVIA Registry Platform (IRP). IQVIA has extensive experience with running large-scale, complex registry projects for surgical and hospital networks, including migration of large data sets from disparate sources.

IQVIA is responsible for all aspects of the technical implementation of platform tasks and updates and support of MOVR.

44. What security reviews has IQVIA had?
IQVIA contracts with a third party to complete a risk analysis per HIPAA requirements.

45. What rights does IQVIA have to the data in MOVR?
IQVIA has access to the site data in MOVR for the purpose of supporting any production issues/questions from the site and managing the registry on behalf of MDA. There are no rights to use the data for any other purpose.

46. What is DNAnexus and what is their role in MOVR?
DNAnexus® has built the world's most secure, trusted cloud platform and global network for scientific collaboration and accelerated discovery. They partner with customers to tackle the world's most exciting opportunities in human health and beyond. MDA has partnered with DNAnexus to

build out the MOVR Visualization & Reporting Platform, or MOVR VRP. This feature of the MOVR study is how sites will assess, organize, manipulate, and report their deidentified MOVR data along with approved requests to utilize the deidentified aggregate data across participating sites.

47. What security reviews has DNAnexus had?
DNAnexus implements measures to ensure the highest level of data security for both research and clinical use. These measures include high-end physical data center security; reliable, replicated data storage; all data is encrypted at rest and in motion; and enterprise and user-controlled permissions for data, analysis tool, and workflow.

The system is continuously monitored (real-time) with incident notification within 1hr of detection. For compliance support, they enable activity and data logging with retention in line with HHS standards, versioned and reproducible analysis tools, and operate in compliance with HIPAA, CAP/CLIA, GCP, 21 CFR Parts 11, and GDPR. The DNAnexus Platform is ISO27001 and HITRUST certified and holds a “Moderate” Authority to Operate for FedRAMP, under the sponsorship of Health & Human Services (HHS).

48. What rights does DNAnexus have to the data in MOVR?
DNAnexus only has access to the deidentified (coded) data in MOVR for the purpose of the visualization platform. They have no access to PHI and no rights to use the deidentified data for any other purpose.

GLOSSARY OF TERMS

Site Participation Agreement (SPA) – the MOVR contract executed between the Institution and the MDA.

Business Associate Agreement (BAA) – governs our relationship under HIPAA and designates the MDA as a business associate with all the rights and responsibilities under HIPAA. The MOVR participation agreement uses the Care Center grant BAA as reference to the existing relationship between an institution and MDA. *The BAA is not required for execution of the SPA/activation in MOVR since it is simply a reference.*

Internal Review Board (IRB) – Ensures that sites and MDA are adequately protecting the patient and that MOVR is being represented accurately.

Informed Consent or Assent Forms (ICFs) – the form the patient (or their guardian) signs acknowledging that they understand MOVR and want to participate.

EHR Integration – a way for data to be pulled from the site's electronic records and pushed into the MOVR data hub.

Case Report Forms (CRF/eCRF) – web-based forms for entering the patient's information and medical data.

Patient Reported Outcome (PRO) – information gathered directly from the patient (not the clinician). Often used to refer to the smartphone app itself where patients will respond to surveys.

Advarra (formerly Quorum) – Advarra is one of two commercial IRBs working with MDA on MOVR. CIRBI is their online portal where sites can submit their IRB documentation for review and check if their approval status is up to date.

WIRB Copernicus Group (WIRB)- WIRB is one of two commercial IRBs working with MDA on MOVR. Connexus is their online portal where sites can submit their IRB documentation for review and check if their approval status is up to date.

IQVIA – a Clinical Research Organization. The software vendor that built the MOVR interface and will support MOVR sites from a technological standpoint.

DNAnexus – a tech company that has developed a secure and compliant cloud platform that is used for the analysis of MOVR data.

Site – Institution (sometimes referred to as Care Center) conducting the MOVR registry.

Site Initiation Visit (SIV) – the 2-hour site training required prior to gaining access to the MOVR platform.

Electronic Health Record (EHR) – the systematized collection of patient and population electronically-stored health information in a digital format. These records can be shared across different health care settings.

Facilities and Administrative Rate (F&A Rate) – the mechanism used to reimburse a site for the infrastructure support costs (indirect costs) associated with sponsored research and other sponsored projects.

Principal Investigator (PI) – a Principal Investigator is the primary individual responsible for the preparation, conduct and administration of a sponsored project in compliance with the applicable laws and regulations and institutional policy governing the conduct of sponsored research.