Evaluating MDA’s MOVR Data Hub as a Source for Real-World Data

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Abstract

About 10 years ago, MDA recognized that there was a significant data shortage regarding disease management decisions. They 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 MO Hub to address this significant shortage and to provide a database for clinicians, drug developers, and regulators with which to make data-driven efficacy and safety decisions. This study describes the mechanisms employed by the MO Hub team to evaluate whether data captured within the hub and compliant with the recent FDA draft guidelines on real-world data (RWD).

Background: MO Hub represents the first data hub that aggregates clinical and genetic data across multiple NMDs, including ALD, BMD, CMD, PSQI, LGMD, Pompe disease, and SMA. Data are collected using electronic case report forms (eCRFs) that capture clinically relevant data for demographics, diagnosis, disease progression, and discontinuation. Last year, the FDA released draft guidelines that focus on using RWD to develop real-world evidence (RWE) to support regulatory decisions. The draft guidance entitled “Assessing Registries to Support Regulatory Decision-Making for Drugs and Biological Products” details what is expected if data registry is used in a submission.

Methods: To evaluate MO Hub compliance with this draft guidance, the MO Hub team turned each criterion stated by the FDA into a question that could be answered “Yes” or “No” to regarding whether MO Hub satisfies the criterion. Questions were transferred to a table with three columns: (1) FDA Guideline, (2) Satisfied by MO Hub, and (3) How MO Hub Satisfies Guideline. With each question occupying its own row, the MO Hub team then identified whether MO Hub satisfies the criterion and provided detailed explanations as to how MO Hub meets the criterion.

Results: The draft guidance mentioned several key topics, including data dictionary, rules for data validations, procedures for data collection, curations, management and storage, data access, data protection, version control, and updating. MO Hub currently meets most of the criteria stated by the FDA, with the MO Hub meeting 77 percent of these criteria (24 out of 31 criteria) and the team is currently implementing strategies to address those that were not satisfied.

Conclusions: As a centralized clinical-entered data hub, analyses demonstrate that MO Hub serves as a research platform that could become an important component of the drug development pipeline for NMDs. MDA is committed to ensuring that MO Hub is compliant with the final guidelines in hopes to help forecast the development of proprietary industry databases and siloing of patient data.

MOVR as a Centralized Data Hub

The MDA launched MO VR to serve as a valuable tool for capturing a longitudinal data that could provide knowledge on disease progression for drug development as well as serve as RWD and RWE in regulatory submissions and post-approval processes. In Fall 2021, the FDA published a draft guidance entitled “Assessing Registries to Support Regulatory Decision-Making for Drugs and Biological Products” that provides a framework for evaluating registries for their use in regulatory decisions.

MO VR’s Compliance with FDA’s Proposed RWE Guidelines

The MO VR team trains MDA Care Centers on data collection and entry processes. Depending on the indication of interest, MO VR team trains MDA Care Centers on data collection and entry processes.

Data Collection and Access

Since its inception in 2019, MO VR has experienced tremendous growth in the number of active care centers participating in the MO VR study, the number of participants providing data to MO VR, and the number of clinical encounters captured in MO VR. MO VR 2021 data report revealed 11,935 encounters and 20 Total Participants.

Conclusions

MO VR satisfies 24 of the 31 guidelines that would be required for the use of MO VR in regulatory submissions according to the draft guidance. For these guidelines that MO VR does not satisfy, the MO VR team is working diligently to develop approaches that would ensure MO VR’s compliance.

The MDA suggests that the FDA create a certification or qualification program that registries can complete to demonstrate that they are FDA-compliant and a reputable source for RWE. This qualification program could allow for registries to prove compliance with the recommendations put forward by this guidance and the other draft guidelines issued under the RWE Program without having to reassert compliance with every product submission, thus greatly reducing the resources needed for both the sponsor and the FDA. With a qualification program, the FDA can be confident in the integrity of the data being submitted, and the focus can be on the data included rather than the processes and procedures used to collect, store, and transform the data.

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Abstract

Goals for MO VR

MOVR aims to serve the entire neuromuscular disease community as the first data hub that will aggregate clinical and patient-reported data for multiple NMDs to improve health outcomes and accelerate drug development. The goals for MO VR are:

1. Gather understanding of the course of disease for specific NMDs.
2. Collect longitudinal patient data that will allow benchmarking of best clinical practices.
3. Use data to develop and implement a clinical quality improvement program for MDA Care Centers across the country.
4. Provide patient-related information about MDA Care Centers and NMD care for families seeking medical care.

Data Dictionary

- Does the registry have a dictionary?
- Is it available to those who need to access the registry data?
- Does it include data elements and the data elements are defined?
- Does it include a structured and coded data elements?
- Does it reference to the source data for the data elements?

Rules for Validations, Data Quality Assessments and Auditing

- Does the registry have rules for the validation of queries and edit checks of registry data?
- Is it made available to those who need to access the registry data?
- 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?
- 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 in the dataset where appropriate)?
- For an electronic database, does the registry ensure data transferred from another data format or system were not altered in the migration process?
- For an electronic database, does the registry explain auditing rules and methods used and the mitigation strategies used to reduce errors?
- Does it describe the types of errors that were identified based on audit findings and the data were corrected?
- Does the registry perform routine descriptive statistical analysis to detect the extent of any missing data, inconsistent data, and data that do not follow up?

Procedures for Data Collection, Curation, Management and Storage

- Does the registry have a defined process and procedures for data collection?
- Does the registry have a defined process and procedures for data curation?
- Does the registry have a defined process and procedures for data management?
- Does the registry have a defined process and procedures for data storage?

Data Access

- Does the registry have a plan for how patients will access and interact with the registry data and the registry’s data governance?
- Does the registry have a plan for how researchers will access and interact with the registry data?
- Does the registry have a plan for how clinicians will access and interact with the registry data?
- Does the registry have terms and conditions for use of the registry data by parties other than the registry owner?
- Does the registry conform to 21 CFR part 11, as applicable, including assurance of access control and audit trails to demonstrate provenance of the registry data and support traceability of the data?

Data Privacy and Security

- Does the registry adhere to applicable (jurisdictional) human subject protection requirements, including protecting the privacy of patient health information?
- Did the registry consult an institutional review board or independent ethical committee for research involving human subject research?
- Does the registry have a plan in place for validating the electronic systems used to collect registry data?

Version Control and Data Consistency

- For an electronic database, does the registry maintain and implement version control by documenting the date, time and originator of data entered in the registry?
- 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 obsolete?
- Are the formats and definitions of the data entered in the registry consistent over time?

Updates to Reflect Changing Clinical Information

- For an electronic database, does the registry account for changes in clinical information over time (such as criteria for disease diagnosis)?
- Are changes in diagnostic criteria or clinical definitions accounted for and documented?

More information about MO VR can be found at mdamovr.org.