MDA's MOVR Data Hub Captures Longitudinal Clinical Data Across 7 Neuromuscular Diseases to Evaluate Current Care Practices and the Impact of FDA-Approved Therapies

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Abstract

Background and Introduction: The Muscular Dystrophy Association (MDA) created the neuroMuscular ObserVational Research Data Hub (MOVR) to address the significant data shortage across neuromuscular diseases (NMDs) and to provide a database for clinicians, drug developers, and regulators with which to make data-driven efficacy and safety decisions around standards of care and disease-modifying therapies. MOVR is powered by the MDA Care Center network, which consists of multidisciplinary care centers across the United States. Currently, MOVR aggregates data from 53 centers and across 7 NMDs, including ALS, BMD, DMD, FSHD, LGMD, Pompe disease, and SMA. These data are collected using electronic case report forms (eCRFs) that capture 31 clinically relevant core data elements for demographics, diagnosis, disease progression, and discontinuation as well as additional data elements that are unique to each indication's diagnostic journey and disease progression.

Objectives: MOVR is in a unique position to not only evaluate current care practices but examine changes in these practices between the pilot dataset (United States Neuromuscular Disease Registry (USNDR) (2013-2018), the MOVR dataset (2019-present) and across care centers.

Methods: Analyses were conducted using data captured within MOVR to understand the data available within the data hub as well as to evaluate changes in care across time for individuals living with DMD and SMA.

Results: MOVR houses data from 4,076 participants and 12,875 clinical encounters, including those collected by MDA's pilot registry (USNDR). Longitudinal data availability ranges from an average of 13.8 ± 14.5 months to 26.4 ± 21.8 months per participant with an average encounter frequency ranging from 4.0 ± 4.2 months to 9.1 ± 7.5 months per participant, depending on the indication. Using these data, we have identified changes over time in medication usage, diagnosis methods, and genetic testing for different indications, suggesting an evolution in care practices. Additionally, data can be compared within the MOVR dataset to measure variability in care from center to center, providing a platform for conservations that begin to understand why variability exists.

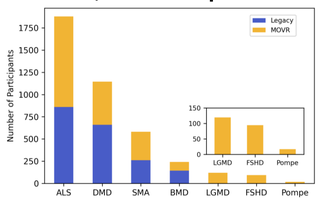
Conclusions: In conclusion, we believe that MOVR is becoming a powerful resource within the neuromuscular disease space by providing unique insights into the current and future care practices for individuals living with these diseases.

MOVR as a Centralized Data Hub

MDA created MOVR to address the significant data shortage in the NMD space. By leveraging the MDA Care Center Network as a source for efficiently capturing clinical data and growing a longitudinal dataset, MOVR is accelerating data collection and its use by researchers, clinicians, and drug developers.

60 Active MOVR Sites

4,076 Participants



Indication	Number of Sites
ALS	33
BMD	43
DMD	48
FSHD	26
LGMD	33
Pompe	12
SMA	46

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.

31

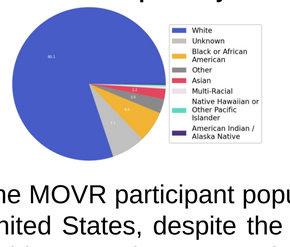
core data elements captured by the electronic Case Report Forms (eCRFs)

Demographics eCRF (During Enrollment)	Diagnosis eCRF* (During Enrollment)	Encounter eCRF* (During Clinical Visits)	Discontinuation eCRF (After End of Study)
Disease Type	Age at Diagnosis	Encounter Date	Date of Discontinuation
Enrollment Date	Age at Symptom Onset	Height	Reason for Discontinuation
Gender	Clinical Diagnosis	Weight	Date of Death
DOB	First Symptoms	Clinical Trial Participation	Cause of Death
Race	Family History	Surgeries	
Ethnicity	Genetic Testing Results	Hospitalizations	
Insurance		Medications	
Education		Pulmonary Devices	
Employment		Assistive Devices	
		Functional Testing	
		Pulmonary Tests	
		Referral Types	

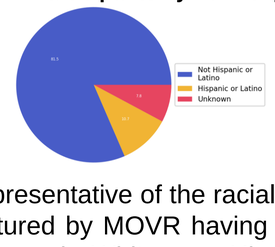
* Diagnosis and Encounter eCRFs contain additional unique fields for each indication.

Participant Demographics

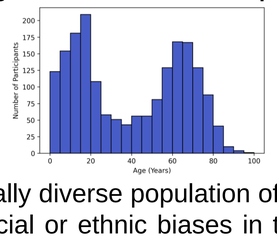
MOVR Participants by Race



MOVR Participants by Ethnicity



Age of Active MOVR Participants



The MOVR participant population is not representative of the racially and ethnically diverse population of the United States, despite the indications captured by MOVR having no known racial or ethnic biases in their incidence. It is not yet clear if the bias towards White, non-Hispanic participants reflects the (1) patient population at MOVR Sites, (2) the selection of patients offered participation in MOVR, and/or (3) inequalities in access to healthcare.

Longitudinal Data Availability and Completeness

Longitudinal data availability is assessed using three different measures, which capture the number of encounters (clinical visits) and the time between those encounters.

Indication	Number of Encounters			Number of Months Between First and Most Recent Encounter			Number of Months Between Consecutive Encounters		
	Count[1]	*Mean*	*StdDev*	*Count*[2]	*Mean*	*StdDev*	*Count*[3]	*Mean*	*StdDev*
ALS	1879	3.3	3.0	1222	13.8	14. 5	4262	4.0	4.2
BMD	241	2.8	2.2	149	25.8	21.1	423	9.1	7.5
DMD	1145	3.4	2.7	813	26.6	21.6	2744	7. 9	6.4
FSHD	94	1.7	1.3	39	14.8	7.9	69	8.4	4.7
LGMD	119	2.1	1.8	60	16.0	19.4	128	7.5	4.9
Pompe	17	1.9	1.1	8	16.3	8. 1	15	8.7	3.5
SMA	581	3.0	2.4	378	23.1	19.1	1158	7.5	6.9

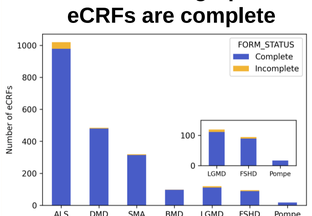
6,919
Legacy Encounters
+
5,956
MOVR Encounters

12,875
Total Encounters

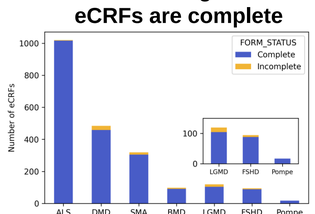
[1]Total number of participants | [2]Number of participants with at least 2 encounters | [3]Number of consecutive encounters

Data completeness is evaluated as the number of forms that are marked complete. Completed forms are those forms that have all required data elements filled out.

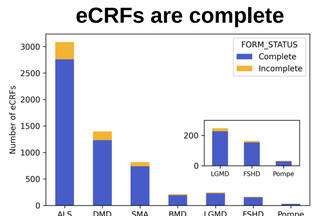
97% of Demographics eCRFs are complete



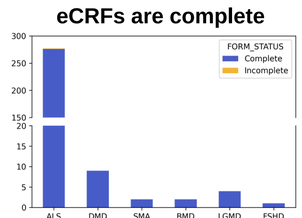
97% of Diagnosis eCRFs are complete



90% of Encounter eCRFs are complete



99% of Discontinuation eCRFs are complete

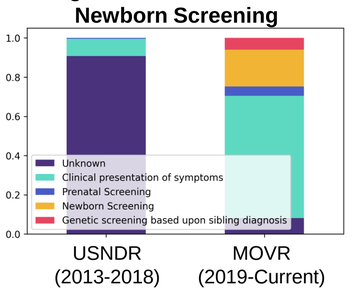


Spinal Muscular Atrophy

Evolution in Care Practices

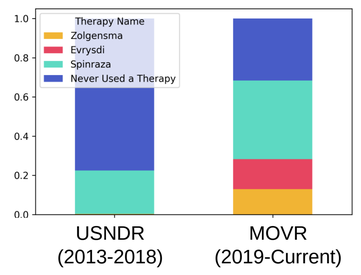
To determine if there have been changes in care practices for individuals living with SMA, we examined 3 data elements in the USNDR dataset (data collection from 2013 to 2018) versus the MOVR dataset (data collection started in 2019).

MOVR Participants are being Diagnosed via Prenatal and Newborn Screening



- Prenatal and newborn screening allows disease-modifying therapies to be administered sooner

MOVR Participants Are Using FDA-Approved Therapies



- With 3 FDA-approved therapies available, more MOVR participants are receiving therapy

Zyprexa Use Among MOVR Participants is Decreased

USNDR (2013-2018)	
zyprexa	69.5%
albuterol	49.6%
spinraza	22.1%
miralax	17.2%
budesonide	8.8%
flonase	6.1%
ranitidine	5.7%
flovent	4.6%
gabapentin	4.6%
acetaminophen	4.2%

MOVR (2019-Current)	
spinraza	45.8%
albuterol	34.8%
zyprexa	18.8%
evrysdi	17.6%
zolgensma	14.7%
multivitamin	11.6%
vitamin d	11.3%
prednisone	9.1%
miralax	8.5%
acetaminophen	8.2%

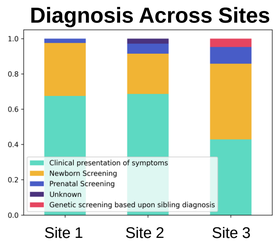
Percent of SMA participants who are / have used medication

- Changes in medication use among MOVR participants suggests that FDA-approved therapies could be changing care requirements

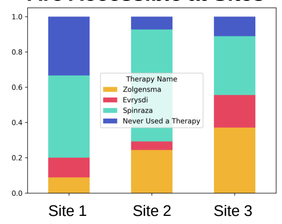
Variability Across Care Centers

To determine if there are differences in care practices across MOVR sites for individuals living with SMA, we examined the same 3 data elements at the 3 MOVR sites that have enrolled the most SMA participants.

NBS is Method of Diagnosis Across Sites



FDA-Approved Therapies Are Accessible at Sites



Albuterol and Zyprexa Use Varies Across Sites

SMA Site 1	
spinraza	52.5%
albuterol	32.5%
prednisone	17.5%
zyprexa	15.0%
budesonide	15.0%
evrysdi	12.5%
zolgensma	10.0%
ativan	5.0%
omeprazole	5.0%
norethindrone	5.0%

SMA Site 2	
spinraza	74.3%
cholecalciferol	42.9%
albuterol	28.6%
zolgensma	28.6%
zyprexa	17.1%
ibuprofen	17.1%
miralax	17.1%
zyrtec	14.3%
clonidine	11.4%
zofran	11.4%

SMA Site 3	
albuterol	85.7%
zolgensma	47.6%
spinraza	42.9%
budesonide	38.1%
prednisolone	33.3%
evrysdi	23.8%
pepcid	23.8%
zyrtec	23.8%
glycolax	23.8%
flonase	19.0%

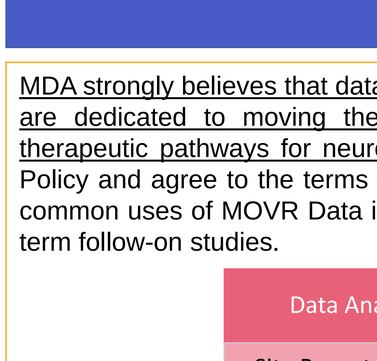
Percent of SMA participants who are / have used medication

Duchenne Muscular Dystrophy

Evolution in Care Practices

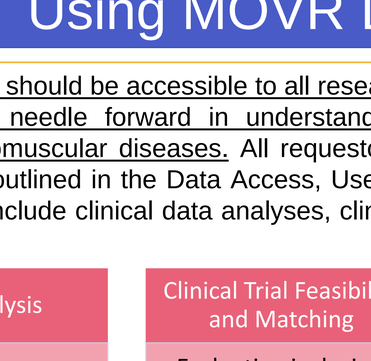
To determine if there have been changes in care practices for individuals living with DMD, we examined steroid and medication usage in the USNDR dataset (data were collected from 2013 to 2018) versus the MOVR dataset (data collection started in 2019).

Deflazacort is More Common Among MOVR Participants



- Steroids continue to be an important aspect of care, but we see a shift away from prednisone to deflazacort in MOVR participants

MOVR Participants Are Starting Steroids Later



- Regardless of steroid type, MOVR participants are starting steroids at an older age

Lisinopril Continues to be at Top Medication in MOVR Participants

USNDR (2013-2018)	
prednisone	36.5%
lisinopril	27.3%
deflazacort	23.2%
albuterol	18.0%
enalapril	12.4%
zantac	9.8%
miralax	8.0%
flonase	5.8%
carvedilol	5.2%
losartan	4.4%

MOVR (2019-Current)	
deflazacort	41.2%
lisinopril	40.2%
vitamin d	30.9%
prednisone	24.1%
multivitamin	19.8%
cholecalciferol	19.0%
albuterol	17.7%
coq10	14.8%
carvedilol	12.4%
miralax	9.5%

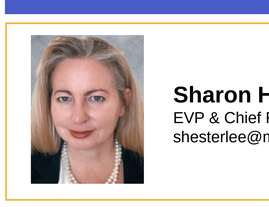
Percent of DMD participants who are / have used medication

- Top medications are relatively similar between USNDR and MOVR participants

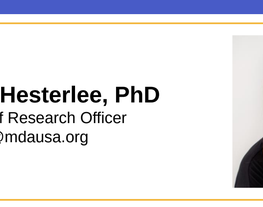
Variability Across Care Centers

To determine if there are differences in care practices across MOVR sites for individuals living with DMD, we examined the same 3 data elements at the 3 MOVR sites that have enrolled the most DMD participants.

Steroid Use is Consistent Across Sites



Steroid Start Age Fluctuates Across Sites



Lisinopril, Vitamin D and Deflazacort are Most Common Across Sites

DMD Site 1	
deflazacort	76.6%
lisinopril	64.9%
vitamin d	62.3%
coq10	61.0%
spironolactone	53.2%
metformin	44.2%
prozac	31.2%
fosamax	28.6%
prednisone	27.3%
multivitamin	27.3%

DMD Site 2	
vitamin d	68.4%
lisinopril	65.8%
solu-cortef	60.5%
prednisone	44.7%
multivitamin	44.7%
deflazacort	39.5%
spironolactone	36.8%
cholecalciferol	26.3%
coq10	26.3%
depotestoterone cypionate	18.4%

DMD Site 3	
cholecalciferol	57.9%
deflazacort	34.2%
lisinopril	28.9%
alendronate	26.3%
hydrocortisone sodium succinate	26.3%
multivitamin	23.7%
losartan	23.7%
vitamin d	23.7%
melatonin	21.1%
famotidine	13.2%

Percent of DMD participants who are / have used medication

Using MOVR Data

MDA strongly believes that data should be accessible to all researchers, clinicians and drug developers who are dedicated to moving the needle forward in understanding disease progression and uncovering therapeutic pathways for neuromuscular diseases. All requestors must follow MOVR's Data Governance Policy and agree to the terms outlined in the Data Access, Use & Distribution Agreement. The three most common uses of MOVR Data include clinical data analyses, clinical trial feasibility and matching, and long-term follow-on studies.

Data Analysis	Clinical Trial Feasibility and Matching	Long-Term Follow-On Studies
• Site Reports • Standards of Care • Cohort Building • Prediction models	• Evaluating inclusion and exclusion criteria • Reducing burden on clinicians	• Real World Data • Efficacy studies

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

Contact the MOVR Team

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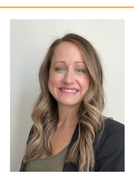

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