

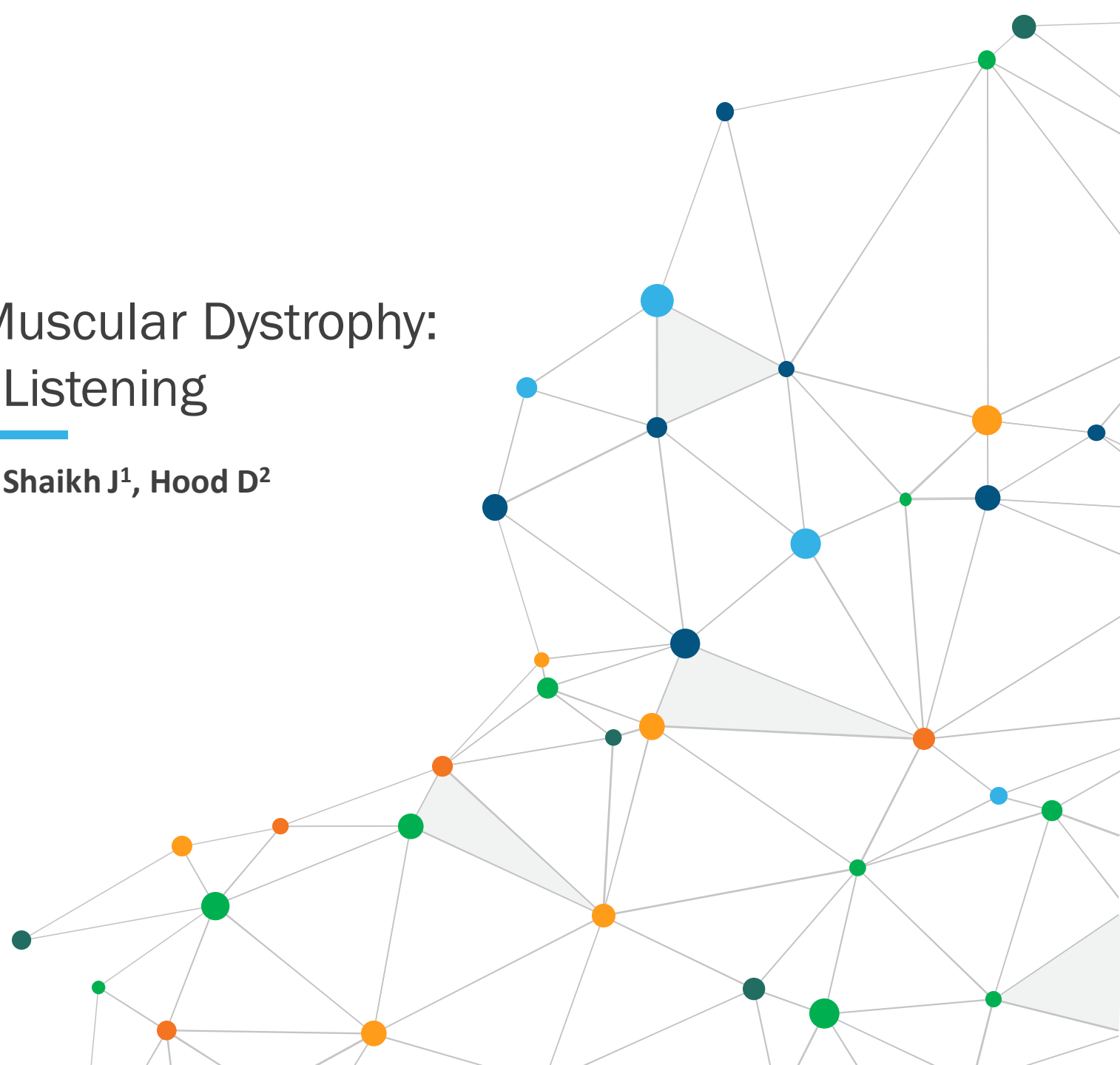


# Patients' Perspectives on Muscular Dystrophy: Insights from Social Media Listening

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## Disclosures

This study was conducted by researchers who are employees of Axtria, Inc. The entirety of the research, including design, execution, and analysis, was funded by Axtria, Inc.

# Introduction

Muscular dystrophy represents a group of hereditary muscular disorders characterized by muscle weakness and atrophy

## Prevalence

Muscular dystrophy affects approximately 3.6 out of every 100,000 people worldwide, with its largest prevalence in the United States at 5.1 in 100,000 people. Duchenne muscular dystrophy is the most common form at 4.8 cases in 100,000 people.<sup>1</sup>



## Impact

There is currently no known cure for muscular dystrophy. The condition often manifests in childhood<sup>2</sup>, reducing life expectancy<sup>3</sup> and quality of life for affected individuals.

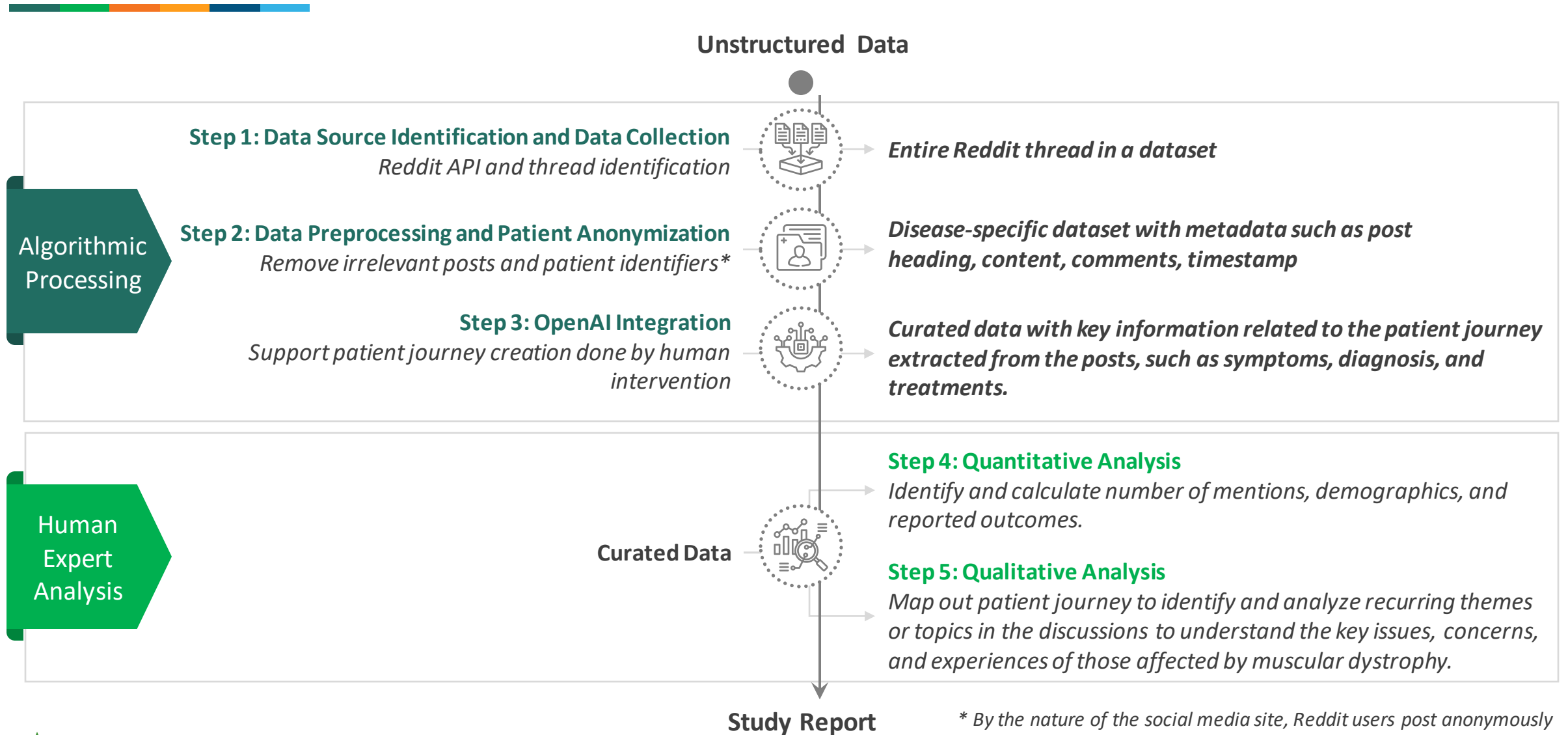
## Research Gaps

Traditional research methods may not capture the nuanced experiences and challenges faced by individuals and families affected by muscular dystrophy.

## Social Media Use

Conducting health research on rare disease patients is difficult due to low prevalence and geographical dispersion. Most of the individuals affected by rare diseases and their caregivers use social media to share experiences, seek information, and find support.<sup>4</sup>

# Methodology



# Audience overview and patient demographics

A total of 802 posts were analyzed from the MD subreddit, with most conversations being from patients

## AUDIENCE OVERVIEW



**400**  
Patients



**124**  
Caregivers



**29**  
Others\*



**249**  
Unidentified

## GENDER DISTRIBUTION (N=240)

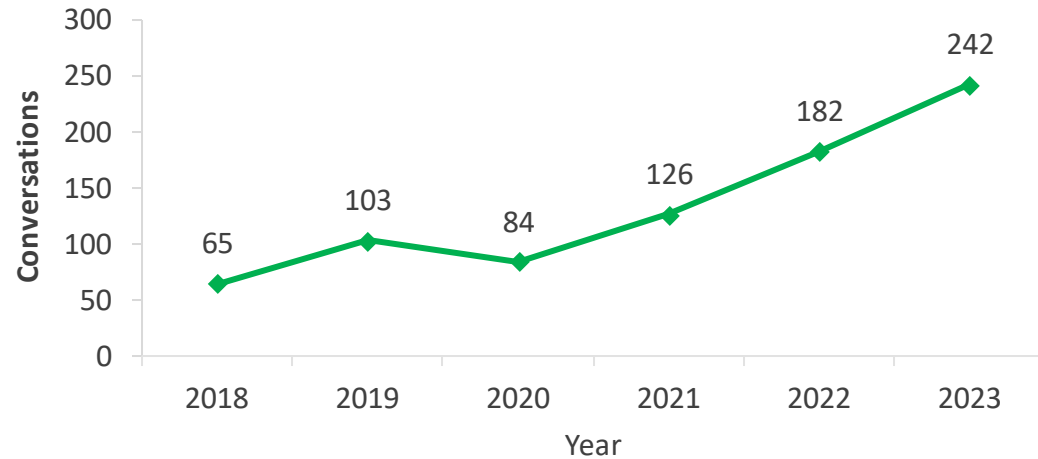


**69%**  
n=166

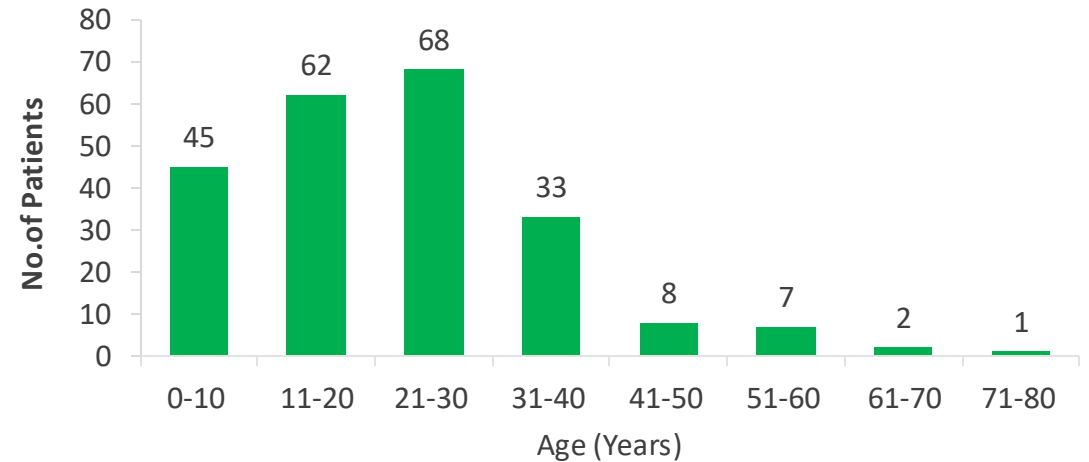


**31%**  
n=74

## Volume Trend (Jan 2018—Dec'2023)

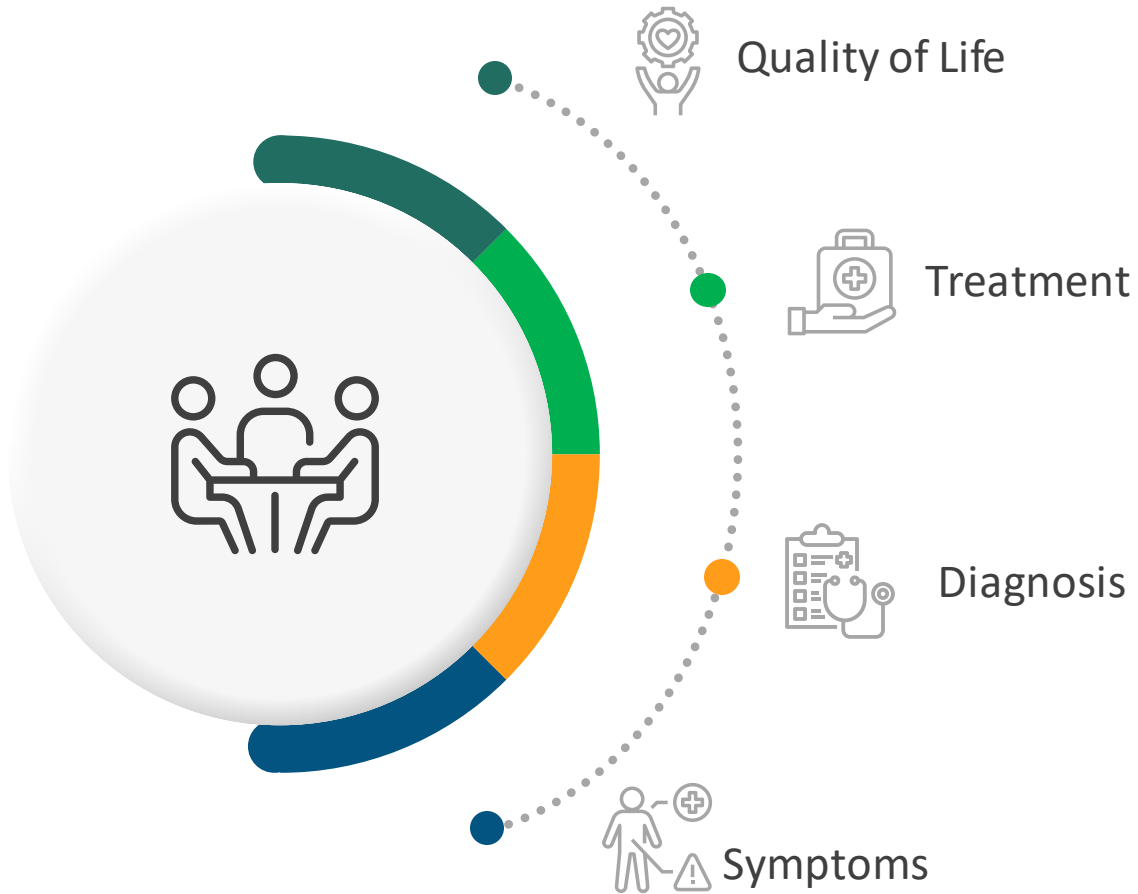


## PATIENT AGE (N=226)

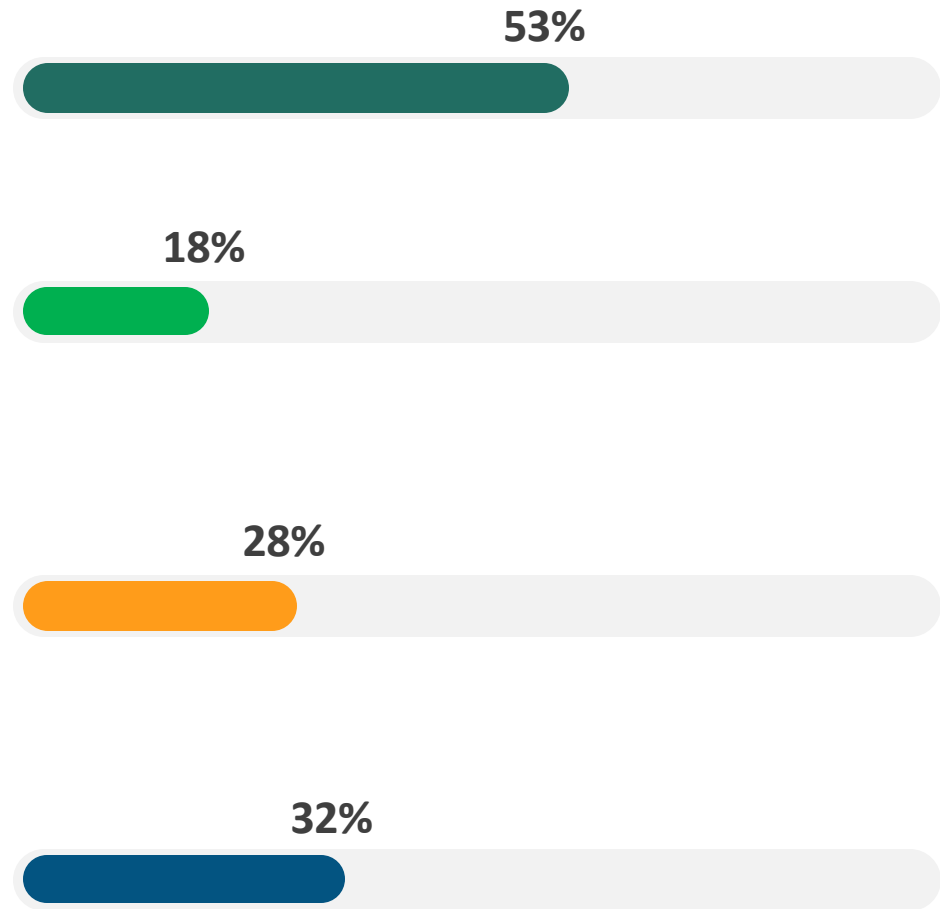


\*Others university students/researchers, companies, communities.

# Primary discussion themes across Reddit were quality of life, followed by symptoms and diagnosis



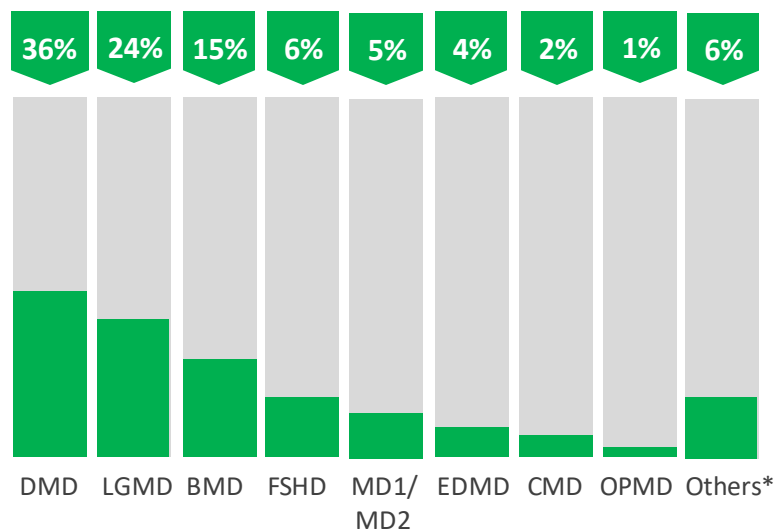
Key Themes Across Reddit (No. of mentions, (%))



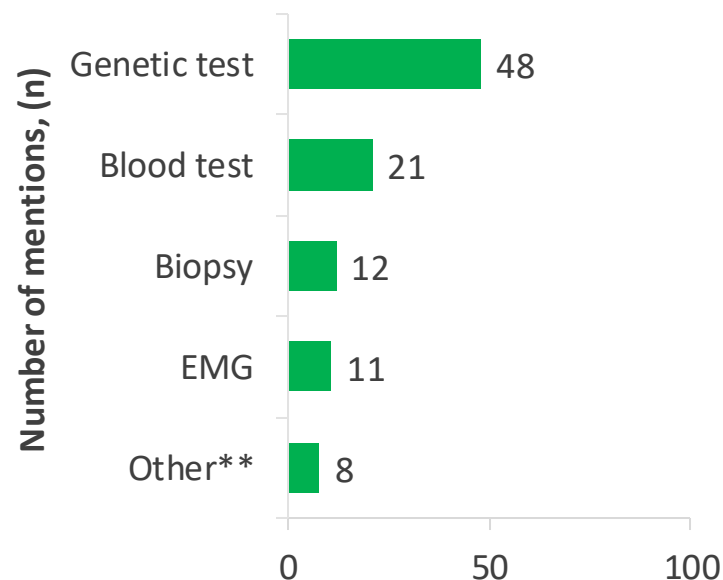
# DMD is frequently reported as the most common type of muscular dystrophy, with genetic testing being the primary diagnostic preference

Patients with muscular dystrophy have reported misdiagnoses, delays in diagnosis, and remaining undiagnosed

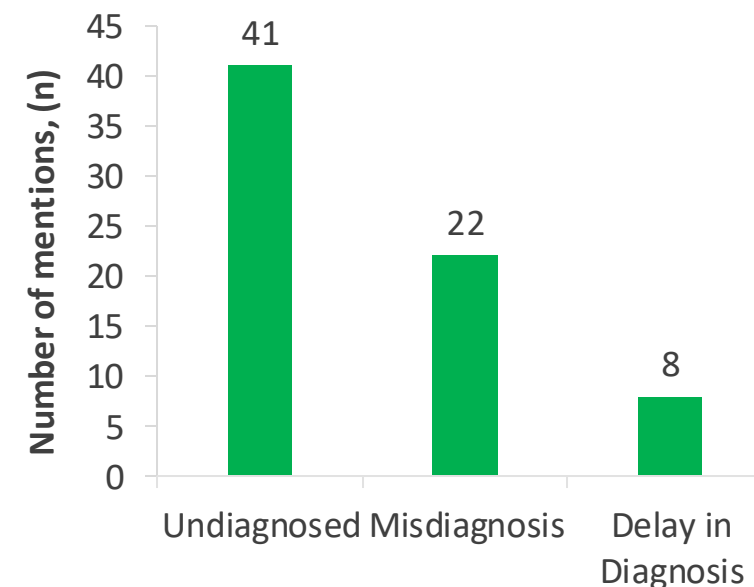
Percentage of posts where the type of muscular dystrophy was reported (n=341)



Diagnostic test (n=73)



Diagnostic challenges in patients with Muscular Dystrophy (n=71)



\*Nemaline Myopathy, Bethlem Myopathy, Desmin myopathy, Miyoshi Myopathy, Mitochondrial Myopathy, myofibrillar myopathy 1, etc.,

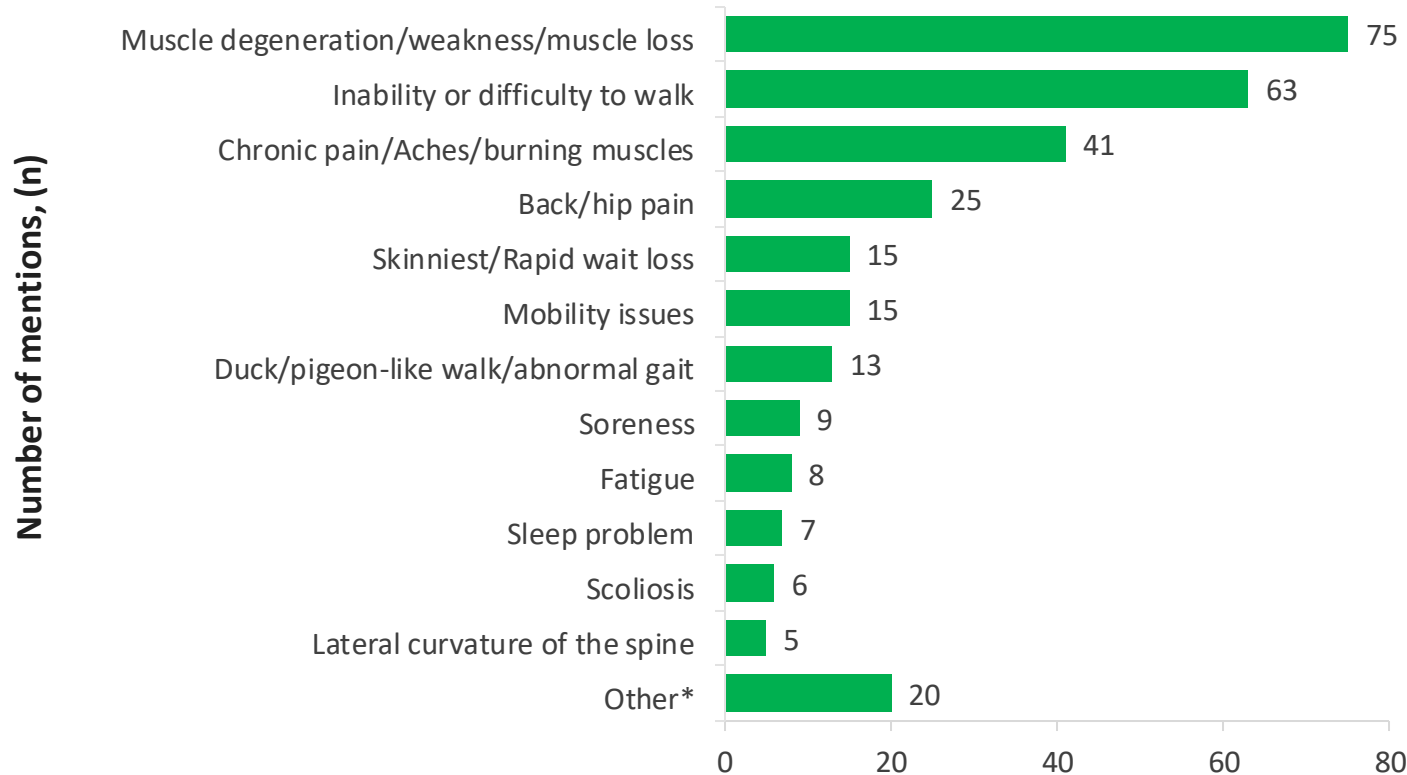
\*\*NCS, MLPA, ECG, X-ray, Myositis antibody test, MRI.

Note: Patients may have reported more than one test.

BMD, Becker muscular dystrophy; CMD, Congenital muscular dystrophy; DMD, Duchenne muscular dystrophy; EDMD, Emery-Dreifuss muscular dystrophy; FSHD, Facioscapulohumeral muscular dystrophy; LGMD, Limb-Girdle Muscular Dystrophy; MD1/MD2, Myotonic dystrophies 1 and 2; OPMD, Oculopharyngeal Muscular Dystrophy

# Commonly discussed symptoms were muscle weakness/degeneration, inability or difficulty walking, and chronic pain which affected daily activities

Symptoms Reported (n=253)



\* Other symptoms include heart problems, breathing problems, migraines, and a dislocated hip, hair thinning, hearing problems  
 n = total number of mentions  
 Note: Patients may have reported more than one symptom.

## Quotes

“ My muscle loss has been very fast in the last year or two. I have lost my ability to walk and stand, and many other things. ”

“ Heavy legs and feeling off balance. Now my gait is waddling a bit. Both hands and arms are affected, with left hand feeling worse (heavier and weaker). ”

“ Due to My extremely curved and twisted spine, almost every activity requires a particular and complicated pose that I experimented with and developed over the last 20 years. ”

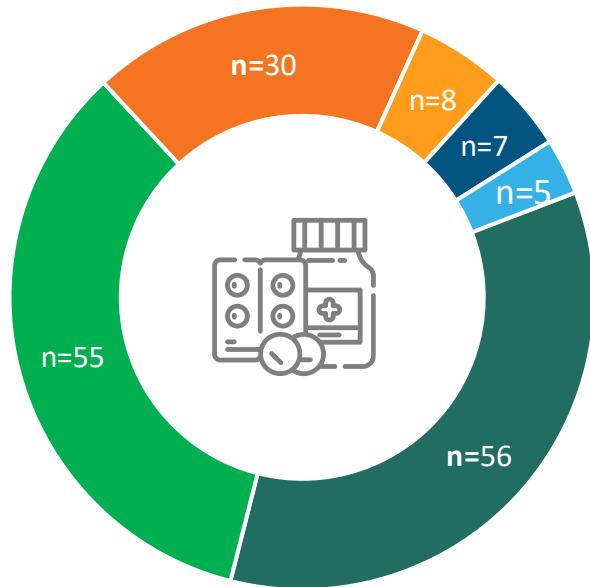
“ My lower back and hips are in constant pain. Like even when I'm sleeping. My back hurts when I go over a bump or anything. ”

“ I slowly walked out, kinda pigeon legged because it hurt too much to walk with straight legs. ”

# Pharmaceutical drugs emerged as the primary pharmacological option for patients with muscular dystrophy

Non-pharmacological treatments such as adaptive devices and physical therapy have been commonly used for managing muscular dystrophy symptoms

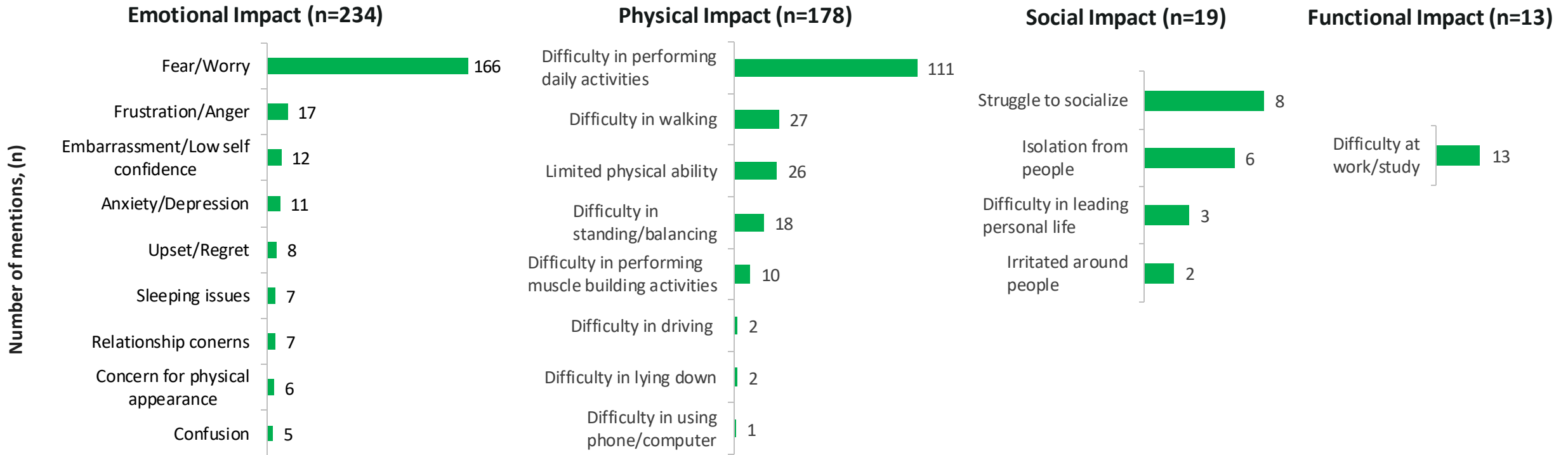
Treatment Options Reported (n=145)



	Corticosteroids	NSAIDs	Narcotic analgesics	Analgesics	Cholinesterase Inhibitor	Anti-Convulsants	Others
Medications	Prednisone, vamorolone	Ibuprofen, anti-inflammatory cream	Cannabis, codeine, methadone	Tylenol®, ibuprofen, kratom, paracetamol	Mestinon®	Gabapentin	Diuretics, muscle relaxers, myostatin, steroids, ASOs (Viltepto®)
Adaptive Devices	Powered wheelchairs, crutches, stilts, walkers, mobility bikes, spinal supports, canes, respiratory support - BiPAP (Bilevel Positive Airway Pressure), ventilators, braces						
Physical Therapy	Exercise, physiotherapy, applying heat/Icy Hot®, massage, water therapy, biofeedback therapy, TENS devices, blood flow resistance training, pool therapy, compression trousers						
Nutritional Supplements	Calcium supplements, multivitamins, cod liver oil, protein shakes						
Surgical Interventions	Placement of defibrillator or pacemaker, spinal fusion/scoliosis correction surgery, total knee replacement surgery						
Gene Therapies	Gene therapy (Elevidys®), exon-skipping therapies, dystrophins, utrophin						

# Muscular dystrophy imposes a significant impact on quality of life

Among various aspects of life, emotional and physical quality of life experienced the most significant negative impact, affecting the daily living of patients.



Quotes

“ I do not know how to describe it, but it's like my leg is not straight but kind of goes off to the side at the knee. I am very scared. Will it get worse? Can I prevent it from getting worse? Will it put me in a wheelchair...? ”

“ I can't drive... hate being driven around & unable to get my own groceries, miss being able to walk everywhere....? ”

“ I get so tired of people who don't have it trying to give me advice. Of course doctors are excluded here but I get tired of people on the street or people like my roommate trying to give me advice about it when she knows absolutely nothing about it ”

“ The isolation, anxiety, denial and despair is crushing.....She left her public school and is barely engaging with online learning. Totally cut off from friends.... ”

“ I work desk job from 9-6 and feel exhausted in just 5-6 hours with pain all over the body. My condition has worsened over the past few months... ”

# Lack of awareness and limited information about the disease, and failure to get a timely and accurate diagnosis emerged as the key unmet needs



## Lack of awareness and limited information about the disease

- Lack of information on symptom management and the absence of education regarding safe and effective treatments
- Limited online resources for disease knowledge; awareness of MD caregiver foundations and education on clinical trial participation



## Diagnosis

- Patients reported enduring years of suffering before receiving a diagnosis
- Patients expressed concerns about misdiagnosis, inaccurate diagnosis, and classification of MD subtype



## Treatment

- Unavailability of treatment providing lasting relief/ lack of effective treatments to manage symptoms
- Limited accessibility and affordability of healthcare options, and restrictive coverage policies



## HCP

- Patients reported lack of trust in HCPs
- Physicians' lack of awareness regarding disease

## Quotes

“ I can't find any help on any other social media MD pages. I can't find what I'm looking for on Google ”

“Just curious if there any other people or parents out there with kids that have this variant? Do you have any leads on upcoming treatments, research, or clinical studies? ”

“ I was recently diagnosed with BMD about a year ago. I had been dealing with it for years but was never diagnosed until recently ”

“I worry the test is not extremely accurate in differentiating one type of MD from another, and that we aren't out of the woods yet ”

“ I read about older drugs that failed and I can imagine people being excited back then. I was reading old posts from when a phase 2 drug got rejected and how sad people were ”

“I'm actively battling health insurance to cover genetic testing ”

“ I have absolutely no trust in Doctors!! Had so many issues with them over the years. I'm worried they are lying to me ”

“My question is, after speaking to the surgeon and the MDA docs near me (both of which seem entirely useless) is it advisable to attempt to surgery to help this? Would it make things worse? ”

# Study Limitations

## Population Bias -

*Bias towards individuals who are active on these platforms, which may not be representative of the entire population.*



**Platform Bias** - *The algorithms that govern content moderation may affect or mask certain data.*

## Temporal Bias -

*Social media data is time-sensitive and may not capture long-term trends or changes in attitudes and behaviors over time.*

## Data Quality



*The quality of data on social media can vary widely, with issues such as misinformation, incomplete information, and ambiguity in posts.*



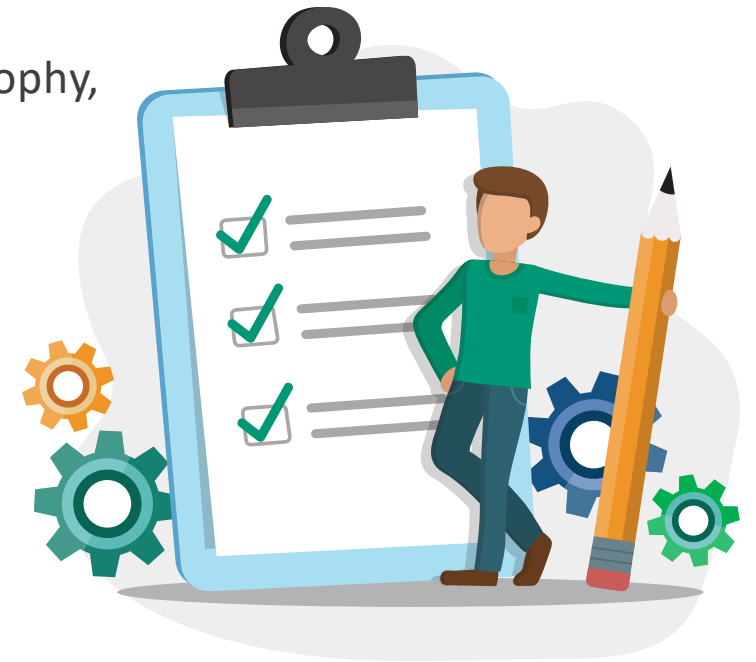
*This study utilized publicly accessible social media data, leading to concerns about the precision of self-diagnosis or patient-reported experiences*



*Symptoms, therapeutic interventions, and self-reported quality of life assessments are derived from aggregated posts that do not clearly represent specific types of MD*

# Summary

- The MD subreddit analyzed 802 conversations, most of which were started by patients and caregivers.
- Patient/caregiver discussions across Reddit platforms were primarily around the quality of life and symptoms.
- DMD is frequently reported as the most common type of muscular dystrophy, with genetic testing being the primary diagnostic preference for patients. In addition, some patients reported experiencing misdiagnosis, delay in diagnosis after multiple rounds of consultation, and remaining undiagnosed.
- Patients reported experiencing a variety of symptoms related to muscular dystrophy, with chronic pain and the inability to walk or difficulty walking affecting the activities of daily living.
- Assessing the way patients wrote about their experiences demonstrated that muscular dystrophy imposed a significant burden of impaired quality of life that spanned multiple aspects of daily life, including patients' emotional and social lives as well as physical and functional well-being.
- Lack of disease awareness, failure to get accurate and timely diagnosis, and limited treatment options were identified as the key unmet needs



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