Facioscapulohumeral Muscular Dystrophy (FSHD) Mobility Aids and Healthcare Utilization After Diagnosis from a Real-World Data Analysis

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An FSHD Caregiver’s Perspective

“We spent a lot of money on medical care and trying to figure out what was wrong... over 25 years. Then we moved, and a young doctor told him to see a neurologist. Otherwise, I doubt we would even know yet.

— CAREGIVER OF 2 FAMILY MEMBERS WITH FSHD*

“It wasn’t until [our son] was diagnosed. [...] The orthopedic surgeon who was supposed to be the best orthopedic surgeon in Columbus for shoulders looked at us like ‘oh my gosh, I should have thought of that’.”

- CAREGIVER OF 2 FSHD FAMILY MEMBERS*

*Data on file: Avidity patient interviews.
Objective

- Describe utilization of medical procedures and mobility devices by patients with Facioscapulohumeral Muscular Dystrophy (FSHD) compared with matched controls (MCs)
- The research being presented today focused on the two years following diagnosis using paid medical claims from IQVIA’s PharMetrics Plus Database.
IQVIA’s PharMetrics® Plus Database

- Retrospective analyses
  - 190+ Million enrollees (who are enrolled in health plans)
  - Data covered the time period from January 2015 through March 2021
  - Closed database allows complete view of patient journey across care settings

- De-identified patient level data

- Data from paid claims in the United States
  - Not an Electronic Medical Record study, so limited to what was coded and paid
  - Patient mix is primarily Commercial PPO & HMO*
  - Limited ‘accuracy / confirmation’ checks in place

- Diagnosis and Hospitalization
  - Every healthcare facility interaction billed through insurance

*PPO (Preferred Provider Organization)/HMO (Health Maintenance Organization) – types of insurance coverage
FSHD Patient Identification

FSHD only uniquely identifiable based on International Classification of Disease, Tenth Revision, Clinical Modification (ICD-10-CM) code G71.02, established November 2018

Patients with FSHD were required to have:
- ≥2 claims specifying diagnoses code [ICD-10-CM=G71.02]
- ≥30 days between claims

Index date: first FSHD claim

Continuous Eligibility for 4 years (48 months):
- ≥2 years (24 months) pre-index
- ≥2 years (24 months) post-index

79 FSHD patients
395 Non-FSHD MCs

First Claim for FSHD
(also known as the index date)

2 years after FSHD diagnosis
Patients to “Matched” Controls (MC)

5 controls without documented muscle disease were matched to each FSHD patient. Controls were matched using R by exact matching with nearest neighbor on:

- Index month
- Baseline age (at index date)
- Region
- Sex
- Plan type
- Payer types

Index month (required 24 months pre- and 24 months post-continuous eligibility)

<table>
<thead>
<tr>
<th>1 FSHD patient</th>
<th>5 non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>RATIO</td>
<td>No FSHD</td>
</tr>
<tr>
<td>≥2 FSHD claims ≥30 days apart</td>
<td>Assigned by matching process</td>
</tr>
<tr>
<td>First diagnosis date</td>
<td>INDEX DATE</td>
</tr>
</tbody>
</table>

Differences between cohorts are considered to be significant when the calculated $P < 0.01$.
Potential Matched Controls (MC) were excluded if they had ICD-10 claims for:

- **Muscular dystrophies**
  - G71.xxx
  - Primary disorders of muscles

- **Muscular wasting**
  - M62.5xx
  - Muscle wasting and atrophy, not elsewhere classified

- **Other muscle disorders**
  - M63.8xx
  - Disorders of muscle in diseases classified elsewhere
Data Definitions

- **Costs** are what was billed and paid for the claim
  - Total of member paid plus plan paid
  - All costs were inflation adjusted to constant 2020 United States dollars

- **Services** represent the chargeable activities per visit

- **Prevalence / Utilization** is the percent of the cohort that had claims for the item

- **Days of Service** represent the number of unique days with a service billed

- **Procedure categories** were based on medical claims with:
  - Healthcare Common Procedure Coding System (HCPCS) or

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Data Reported Are Per-Member-Per-Year for Costs, Number of Services and Days of Service
Baseline Comparisons Between Cohorts

- There were 79 patients with FSHD and 395 MCs that met the study criteria.
- All comparisons between cohorts (FSHD vs MCs) non-significant ($P > 0.05$) for the matched criteria.
- In the US, Medicaid typically covers low-income individuals, Medicare typically covers those $\geq 65$ years old and special needs populations (which can include FSHD).

### Demographics

<table>
<thead>
<tr>
<th>Descriptive Characteristics</th>
<th>FSHD Patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age in years, Mean (Standard Deviation)</td>
<td>47.9 (17.9)</td>
<td>48.4 (17.5)</td>
</tr>
<tr>
<td>Age $&lt;$ 18</td>
<td>6.3%</td>
<td>5.3%</td>
</tr>
<tr>
<td>18 $\leq$ Age $&lt;$ 65</td>
<td>81.0%</td>
<td>82.0%</td>
</tr>
<tr>
<td>65 $\leq$ Age</td>
<td>12.7%</td>
<td>12.7%</td>
</tr>
<tr>
<td>Female</td>
<td>43.0%</td>
<td>44.3%</td>
</tr>
</tbody>
</table>

### Plan Type

<table>
<thead>
<tr>
<th>Plan Type</th>
<th>FSHD Patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Preferred Provider Organization</td>
<td>81.0%</td>
<td>79.7%</td>
</tr>
<tr>
<td>Health Maintenance Organization</td>
<td>12.7%</td>
<td>12.9%</td>
</tr>
<tr>
<td>Point of Service Plan</td>
<td>3.8%</td>
<td>3.8%</td>
</tr>
<tr>
<td>Consumer Directed Health Care</td>
<td>2.5%</td>
<td>3.5%</td>
</tr>
</tbody>
</table>

### Payer Type

<table>
<thead>
<tr>
<th>Payer Type</th>
<th>FSHD Patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Commercial</td>
<td>60.8%</td>
<td>63.0%</td>
</tr>
<tr>
<td>Self-Insured</td>
<td>31.6%</td>
<td>30.6%</td>
</tr>
<tr>
<td>Medicare Advantage</td>
<td>5.1%</td>
<td>3.8%</td>
</tr>
<tr>
<td>Medicare Supplemental</td>
<td>2.5%</td>
<td>2.5%</td>
</tr>
</tbody>
</table>
FSHD Patients Have More Comorbidity Burden Than MCs

### Charlson Comorbidity Index

<table>
<thead>
<tr>
<th>Descriptive Characteristics</th>
<th>FSHD Patients (N=79)</th>
<th>Non-FSHD MCs (N=395)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Mean or Percent</td>
<td>Standard Deviation</td>
</tr>
<tr>
<td>Pre-index Charlson Comorbidity Index*</td>
<td>1.13</td>
<td>1.65</td>
</tr>
<tr>
<td>Percent with Pre-index CCI&gt;1</td>
<td>27.8%</td>
<td></td>
</tr>
<tr>
<td>Post-index Charlson Comorbidity Index*</td>
<td>1.37</td>
<td>2.13</td>
</tr>
<tr>
<td>Percent with Post-index CCI&gt;1*</td>
<td>29.1%</td>
<td></td>
</tr>
</tbody>
</table>

**Number of ICD-10 Diagnoses**

- Pre-Index Period:
  - FSHD patients: 10.27
  - Non-FSHD MCs: 6.39

- Post-Index Period:
  - FSHD patients: 22.62
  - Non-FSHD MCs: 10.97

*P<0.05

Charlson Comorbidity Index – index to estimate the risk of death with specific comorbidity conditions

Data on file: Avidity Biosciences, Inc.; San Diego, CA
Healthcare Utilization During the Study Period was Significantly Higher in FSHD Patients

Compared with MCs, patients with FSHD had more PMPY overall utilization, costs, and services

- 44 procedures were significantly more prevalent after a diagnosis

Between cohort comparisons for all metrics, $P<0.0001$
Healthcare Services After Index Date are Higher in Patients with FSHD

Costs also continue to increase for patients with FSHD

### Higher Average Cost:

<table>
<thead>
<tr>
<th>Service</th>
<th>FSHD patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Emergency Department</td>
<td>$44</td>
<td>$2,166*</td>
</tr>
<tr>
<td>Inpatient</td>
<td>$68</td>
<td>$1,618</td>
</tr>
<tr>
<td>Lab</td>
<td>$5,803*</td>
<td>$2,393</td>
</tr>
<tr>
<td>Office</td>
<td>13.4%</td>
<td>42.8%</td>
</tr>
<tr>
<td>Other</td>
<td>100.0%</td>
<td>94.9%</td>
</tr>
<tr>
<td>Outpatient</td>
<td>34.9%</td>
<td>60.0%</td>
</tr>
</tbody>
</table>

*P > 0.01
Data on file: Avidity Biosciences, Inc.; San Diego, CA
Patients With FSHD Required More Mobility Related Procedures and Durable Medical Equipment

Services are mostly related to physical therapy, transportation, and supplies

Data on file: Avidity Biosciences, Inc.; San Diego, CA
FSHD Patients Experience Higher Prescription Burdens During the Post-Index Period

**Percent of cohort with claims for prescription drugs**

- **FSHD patients:** 92.41%
- **Non-FSHD MCs:** 80.51%

**Cost of prescription drugs**

- **FSHD patients:** $4,844
- **Non-FSHD MCs:** $2,734

*P > 0.01
Data on file: Avidity Biosciences, Inc.; San Diego, CA
FSHD Patients Experience Higher Prescription Burdens During the Post-Index Period

Average number of prescription fills

<table>
<thead>
<tr>
<th></th>
<th>FSHD patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Average</td>
<td>35.20</td>
<td>20.31</td>
</tr>
<tr>
<td>Range</td>
<td>14.90</td>
<td>5.98</td>
</tr>
</tbody>
</table>

Average number of drugs per patient

<table>
<thead>
<tr>
<th></th>
<th>FSHD patients</th>
<th>Non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Average</td>
<td>12.53</td>
<td>6.55</td>
</tr>
<tr>
<td>Range</td>
<td>5.98</td>
<td></td>
</tr>
</tbody>
</table>
Patients With FSHD Have More Surgical Interventions During the Post-index Period

Integumentary (Skin related) interventions topped the list, followed by other musculoskeletal

35.3% types of surgery were more frequently needed by patients with FSHD compared with non-FSHD individuals

*P=0.02
Data on file: Avidity Biosciences, Inc.; San Diego, CA
FSHD Patients Required More Comorbidity Evaluations than MCs During the Post-index Period

- Other significant non-mobility procedures with greater utilization in the FSHD cohort

![Bar Chart]

Data on file: Avidity Biosciences, Inc.; San Diego, CA

*P<0.01, †P=0.02
Conclusions

- Patients with FSHD have higher healthcare utilization overall, for mobility-related procedures and durable medical equipment, than MCs after diagnosis.
  - This illustrates the significant challenges relating to ambulation and the associated financial burden
- FSHD is a complicated neuromuscular disorder, affects multiple systems, and requires more procedures with higher costs and services.
- Based on the high unmet need for effective treatments for FSHD, Avidity is developing AOC 1020 a first in class AOC targeting the inappropriate expression of DUX4, the underlying cause of muscle degeneration in FSHD
Thank You

- To access this, and other research conducted by the Avidity team, please use the QR code below or go to www.aviditybiosciences.com/platform/publications/
Backup
The FSHD Patient Journey
The journey begins before diagnosis and continues throughout their lifetime

About FSHD – Learn what causes this neuromuscular disorder and the common symptoms

Diagnosis and Treatment Options – Recognize the characteristic clinical signs of FSHD and its associated treatment

Before Diagnosis – Understand what patients with FSHD experience before formal diagnosis

After Diagnosis – Understand what patients with FSHD experience after formal diagnosis

The Future – Discover what Avidity Biosciences is doing to potentially change the outlook for patients with FSHD
An FSHD Patient’s Perspective

After diagnosis, healthcare costs continue to significantly increase

“Medical care you need isn’t always covered by insurance. That means more out-of-pocket expenses for you, and it only increases as your disability increases.”

— PATIENT WITH FSHD*
After Diagnosis Summary

The burden continues to increase as the disease progresses

- Use more prescription medications
- Have a higher burden of disease, risk of death, and more symptoms
- Use more assistive devices
- Have more and different diagnostic, lab tests, radiology exams
- Have numerous and different comorbidities
- Use more clinician-administered medications such as injectables
- See physicians and pharmacists more often
- Have higher healthcare costs
- Use more outpatient, office, and other care*

*Assisted living facility, home, intermediate-care facility.

Data on file: Avidity Biosciences, Inc.; San Diego, CA
## Lung Function and Other Chemistry

### Lung Function

<table>
<thead>
<tr>
<th>CPT</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>94101</td>
<td>Breathing capacity test</td>
</tr>
<tr>
<td>94102</td>
<td>Up to 2 yrs old, spirometry</td>
</tr>
<tr>
<td>94103</td>
<td>= 2 yrs, spirometry w/dilator</td>
</tr>
<tr>
<td>94104</td>
<td>= 2 yrs, lung volumes</td>
</tr>
<tr>
<td>94105</td>
<td>Patient recorded spirometry</td>
</tr>
<tr>
<td>94106</td>
<td>Patient recorded spirometry</td>
</tr>
<tr>
<td>94107</td>
<td>Review patient spirometry</td>
</tr>
<tr>
<td>94108</td>
<td>Evaluation of wheezing</td>
</tr>
<tr>
<td>94109</td>
<td>Evaluation of wheezing</td>
</tr>
<tr>
<td>94110</td>
<td>Lung function test (mbc/mv)</td>
</tr>
<tr>
<td>94111</td>
<td>Residual lung capacity</td>
</tr>
<tr>
<td>94112</td>
<td>Expired gas collection</td>
</tr>
<tr>
<td>94113</td>
<td>Thoracic gas volume</td>
</tr>
<tr>
<td>94114</td>
<td>Lung nitrogen washout curve</td>
</tr>
<tr>
<td>94115</td>
<td>Measure airflow resistance</td>
</tr>
<tr>
<td>94116</td>
<td>Breath airflow closing volume</td>
</tr>
<tr>
<td>94117</td>
<td>Respiratory flow volume loop</td>
</tr>
<tr>
<td>94118</td>
<td>Co2 breathing response curve</td>
</tr>
<tr>
<td>94119</td>
<td>Hypoxia response curve</td>
</tr>
</tbody>
</table>

### Other Chemistry

- 082 Other chemistry in the category group: Pathology and Laboratory
- Includes HCPC/CPT codes: 82000–84999
  - Per design, this excludes:
    - Dysferlin protein blood test (in DM genetic list)
    - Muscle enzyme test

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Patients With FSHD Have More Hospital Admissions After Diagnosis

The most common admissions are other skin disorders, respiratory issues, and surgical complications.

- **Other Skin Disorders**
  - >5% Higher
  - FSHD: 5.1%
  - Non-FSHD: 0.0%

- **Pleurisy, pneumothorax, pulmonary collapse**
  - >5% Higher
  - FSHD: 6.3%
  - Non-FSHD: 0.5%

- **Complications of Surgical Procedures and Medical Care**
  - >3% Higher
  - FSHD: 3.8%
  - Non-FSHD: 0.0%

- **Acute cerebrovascular disease**
  - >3% Higher
  - FSHD: 3.8%
  - Non-FSHD: 0.0%

All P<0.0001
Data on file: Avidity Biosciences, Inc.; San Diego, CA