Prescription Medication Use Prior to and Following a Diagnosis of Facioscapulohumeral Muscular Dystrophy (FSHD): Learnings from the Patient Journey

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Background
• FSHD is a rare, slowly progressive, genetic skeletal muscle disease. Muscle weakness usually presents in the face and upper extremities, eventually extending to the trunk and lower body.1,2
• FSHD is one of the most common forms of muscular dystrophy affecting approximately 18,000-38,000 people in the US.1,2
• FSHD is caused by the aberrant expression of the DUX4 transcription factor in skeletal muscle.
• Patients experience significant physical limitations, pain, fatigue, and an overall negative impact on wellbeing.3,4
• Real-world data characterizing the FSHD patient journey and specialty pharmacy products used are limited.
• Currently there are no approved disease modifying therapies for FSHD, and medical treatment is focused on symptom management.5

Objective
• To describe the changes in overall healthcare and prescription utilization and examine the use of specialty pharmacy products two years pre- and two years post-diagnosis of FSHD.

Study Design and Methods
• We used PharMetrics deidentified U.S. claims (Jan 2015—Mar 2021) to retrospectively evaluate care for:

<table>
<thead>
<tr>
<th>1 FSHD patient</th>
<th>5 non-FSHD MCs</th>
</tr>
</thead>
<tbody>
<tr>
<td>22 FSHD claims</td>
<td>230 days apart</td>
</tr>
<tr>
<td>First diagnosis date</td>
<td>INDEX DATE</td>
</tr>
</tbody>
</table>

• Cohorts were matched on index month, baseline age, region, gender, plan, and payer types.
• All patients and controls had continuous data for two years before and two years after their index date.
• Specialty pharmacy product use was analyzed in the periods before and after diagnosis.
• Because data were from claims, diagnosis for multi-indication drugs were not definitive.
• Drug categories were reported based on the FDA label.
• Data reported are per-member-per-year for costs, number of services and days of service.
• Costs were adjusted to 2020 U.S. dollars.
• All reported finding significant P<0.05 unless noted.

Results
• We identified 79 patients with FSHD and 395 MCs.

### Total per member per year medical and prescription costs

<table>
<thead>
<tr>
<th></th>
<th>Pre-diagnosis</th>
<th>Post-diagnosis</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Pre-index-Post-index</th>
</tr>
</thead>
<tbody>
<tr>
<td>FSHD</td>
<td>$4,752</td>
<td>$5,231</td>
<td>$10,000</td>
<td>$10,000</td>
<td>$0</td>
</tr>
<tr>
<td>MCs</td>
<td>$4,800</td>
<td>$4,844</td>
<td>$2,000</td>
<td>$2,013</td>
<td>$44</td>
</tr>
</tbody>
</table>

### Total prescription costs

<table>
<thead>
<tr>
<th></th>
<th>Pre-diagnosis</th>
<th>Post-diagnosis</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Pre-index-Post-index</th>
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<td>$10,000</td>
<td>$10,000</td>
<td>$3,000</td>
</tr>
<tr>
<td>MCs</td>
<td>$3,000</td>
<td>$3,000</td>
<td>$479</td>
<td>$479</td>
<td>$0</td>
</tr>
</tbody>
</table>

### # of prescription products

<table>
<thead>
<tr>
<th></th>
<th>Pre-diagnosis</th>
<th>Post-diagnosis</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Pre-index-Post-index</th>
</tr>
</thead>
<tbody>
<tr>
<td>FSHD</td>
<td>23</td>
<td>24</td>
<td>10</td>
<td>10</td>
<td>1</td>
</tr>
<tr>
<td>MCs</td>
<td>22</td>
<td>22</td>
<td>10</td>
<td>10</td>
<td>0</td>
</tr>
</tbody>
</table>

### # of prescriptions filled

<table>
<thead>
<tr>
<th></th>
<th>Pre-diagnosis</th>
<th>Post-diagnosis</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Pre-index-Post-index</th>
</tr>
</thead>
<tbody>
<tr>
<td>FSHD</td>
<td>23</td>
<td>24</td>
<td>10</td>
<td>10</td>
<td>1</td>
</tr>
<tr>
<td>MCs</td>
<td>22</td>
<td>22</td>
<td>10</td>
<td>10</td>
<td>0</td>
</tr>
</tbody>
</table>

### Days with prescription claims

<table>
<thead>
<tr>
<th></th>
<th>Pre-diagnosis</th>
<th>Post-diagnosis</th>
<th>Pre-index</th>
<th>Post-index</th>
<th>Pre-index-Post-index</th>
</tr>
</thead>
<tbody>
<tr>
<td>FSHD</td>
<td>44</td>
<td>45</td>
<td>10</td>
<td>10</td>
<td>1</td>
</tr>
<tr>
<td>MCs</td>
<td>44</td>
<td>45</td>
<td>10</td>
<td>10</td>
<td>0</td>
</tr>
</tbody>
</table>

### Summary

- Pre-diagnosis, patients with FSHD filled specialty pharmacy products for:
  - Cardiovascular, infectious diseases, psychiatric, skin, allergy, ocular, and gastrointestinal conditions.
  - Analgesics, muscle relaxants, steroids, and hormone therapies.
  - Products with FDA indications for various autoimmune conditions.

- Specialty pharmacy products used by the FSHD cohort prior to a definitive diagnosis included:
  - AOC 1020, a first-in-class antibody oligonucleotide conjugate targeting DUX4, the underlying cause of FSHD.
  - The U.S. Food and Drug Administration (FDA) has granted fast track designation to AOC 1020 for the treatment of FSHD.

Results (continued)
• Post-diagnosis, patients with FSHD:
  - Continued or expanded use of agents for cardiovascular, infectious, psychiatric, ocular, and gastrointestinal conditions.
  - They also used analgesics, muscle relaxants, hormone therapies, steroids, diabetes agents, and drugs with autoimmune indications.
  - Initiated drugs for age-related macular degeneration, overactive bladder, and osteoporosis.
  - Post-index specialty pharmacy drugs used by the FSHD cohort included:
    - emtricitabine, tenofovir alafenamide and bictegravir.
    - dronedarone.

Conclusions
• Patients with FSHD have higher healthcare utilization than MCs before and after diagnosis.
• FSHD is a complicated neuromuscular disorder, affects multiple systems, and requires various medications, more prescriptions, and more days with prescription claims.
• Based on the high unmet need for novel targeted treatments, Avidity Biosciences is developing AOC 1020, a first-in-class antibody oligonucleotide conjugate targeting DUX4, the underlying cause of FSHD.
• The U.S. Food and Drug Administration (FDA) has granted fast track designation to AOC 1020 for the treatment of FSHD.

References
6. IVI-BASE. Available at https://www.biosciences.com/guides/passport/drug-names