Understanding the Patients’ Journey Pre- and Post-Diagnosis of Facioscapulohumeral Muscular Dystrophy (FSHD): a Real-World Retrospective Data Analysis

Chamindra Konermsan, MD1; Kathryn A. Munoz, PhD, MPH2; Richard A. Brook, MS, MBA3; Nathan L. Kleinman, PhD1; Kelly DiTrapani, BA, BSN1; Bradley McEvoy, DrPH1; Alissa N. Peters1; Chao-Yin Chen, PhD1; Mark C. Stahl, MD, PhD1

1University of California San Diego; 2Avidity Biosciences, Inc.; 3Better Health Worldwide. All authors have met authorship criteria.

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Objectives

Describe the changes in outcomes (healthcare conditions, services, costs, and care days) for patients with FSHD compared with matched controls (MCs) 2 years post-diagnosis versus 2 years pre-diagnosis.

Methods

- Retrospective database analysis to compare outcomes for patients with FSHD versus MCs
- The FSHD cohort is defined as having ≥2 FSHD claims ≥30 days apart
- Claims identified by International Classification of Disease Tenth Revision (ICD-10) code G71.02
- The first diagnosis date was used for the index date
- FSHD patients were matched to a 5% random sample of eligible non-FSHD controls
- Matching was done using R’s MatchIt procedure, with nearest neighbor matching (exact matching on month of index date)
- Cohorts were matched (5-MC:1-FSHD) on index month and baseline age, region, gender, plan, and payer type
- All subjects (patients and MCs) had a minimum of 48 months of continuous data
- 24-months prior to their index date
- 24 months following (post “) their index date (includes the index date)
- The index date was the beginning of the post-index evaluation period

Results

- We identified 79 FSHD patients and 395 MCs
- There were no significant differences between cohorts for age, gender, US region, patient plan type, and patient insurance type (Table 1)
- Among the FSHD cohort, the following locations of care had a significant (p<0.05) increase in post- minus pre-diagnosis:
  - The difference in percent of patients seeing the Emergency Department was 16.5% (primarily due to increases in respiratory condition visits)
  - Number of days and total medical days increased by 6.0 days (also significantly greater than the 0.5-month increase in the MC cohort)
  - Total annual medical costs per person increased $5,646
- FSHD patients had more comorbidities compared to MCs:
  - Prevalence increased significantly in 5 AHRQ categories (Figure 1)
  - Costs charged significantly in 3 AHRQ categories (Figure 2)
- Number of services per person increased significantly in 3 AHRQ categories (Figure 3)

Conclusions

- Healthcare utilization increased significantly in FSHD patients following diagnosis and was higher both overall and in different categories than in MCs
- This likely reflects the need to investigate and manage previously unsuspected manifestations of FSHD following formal diagnosis
- Future research should confirm if these findings hold true in longer-term follow-up
- These data highlight the high unmet needs for FSHD patients, including higher costs, more days of care, and more prevalent and costly comorbidity management and the need for novel targeted treatments

- Based on the high unmet need, Avidity Biosciences is planning clinical trials with a first-in-class antibody oligonucleotide conjugate targeting DUX4, the underlying cause of FSHD, in 2022


Table 1: Age, US Region, Insurance, and Payer Types Were Similar Between FSHD Patients and MCs

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>FSHD Patients (N=79)</th>
<th>MCs (N=395)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, mean (SD) years</td>
<td>47.9 (17.9)</td>
<td>47.9 (17.9)</td>
</tr>
<tr>
<td>US region</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Midwest</td>
<td>56%</td>
<td>56%</td>
</tr>
<tr>
<td>West</td>
<td>16.9%</td>
<td>16.9%</td>
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<tr>
<td>Insurance type</td>
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<tr>
<td>Preferred provider organization</td>
<td>67%</td>
<td>67%</td>
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<tr>
<td>Governmental organization</td>
<td>14%</td>
<td>14%</td>
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<tr>
<td>Point of service plan</td>
<td>19%</td>
<td>19%</td>
</tr>
<tr>
<td>Payer type</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Medicare</td>
<td>27%</td>
<td>27%</td>
</tr>
</tbody>
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Abbreviations:

References: