Sex-related Utilization Differences in the 12-months After a Diagnosis of Facioscapulohumeral Muscular Dystrophy (FSHD)

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Objective

- To identify differences between male (M-) and female (F-) patients with FSHD and their matched controls (MCs)

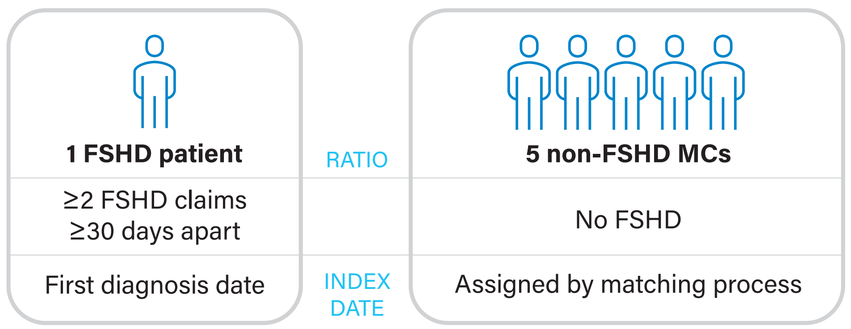
Background

- FSHD is a rare, variable 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 16,000–38,000 people in the US[1,2]
- FSHD is caused by the aberrant expression of the DUX4 transcription factor in skeletal muscle[1,2]
- Patients experience significant physical limitations, pain, fatigue, and an overall negative impact on wellbeing[3,4]
- Sex-specific data on patients with FSHD are limited
- Currently there are no approved disease-modifying therapies for FSHD, and medical treatment is focused on symptom management[5]

Design/Methods

- We used PharMetrics de-identified US claims (Jan 2015—Mar 2021) to retrospectively evaluate care for FSHD and MC cohorts (Figure 1)
  - The FSHD cohort is defined as having ≥2 FSHD claims ≥30 days apart. FSHD claims were identified by International Classification of Disease Tenth Revision (ICD-10) code G71.02
  - The FSHD ICD-10 code was implemented in 2018. The pre-2018 period was used to exclude MCs with claims for muscular dystrophies (ICD-10-CM G71.x xx), muscular wasting (ICD-10-CM M62.5xx), or other muscle disorders (ICD-10-CM M63.8xx)

Figure 1: Cohort Identification



- Cohorts were matched on index month, baseline age, region, sex, plan, and payer types
- All subjects had >12 months of continuous data following their index date
- Costs are the total of member paid plus plan paid. All cost data were adjusted to constant 2020 US dollars
- Comorbidities were classified by the Agency for Healthcare Research and Quality (AHRQ) specific categories
- Prescription products classified by the Anatomical Therapeutic Chemical (ATC). The ATC3 classification data presented in this poster are based on chemical substance
- Services represent the chargeable activities per visit
- Data reported are per-member-per-year for cost and number of services
- All reported findings are statistically significant (p≤0.001) unless noted
  - P values for prevalence and utilization comparisons are based on chi-square tests the percent of the cohort
  - P values for cost and number of services are based on *t*-tests

Results

- We identified 280 individuals with FSHD (male=155, female=125) and 1400 MCs (male=774, female=626), with no significant baseline characteristics differences between male and female cohorts (FSHD and MCs) (Table 1)

Table 1: Descriptive Characteristics*

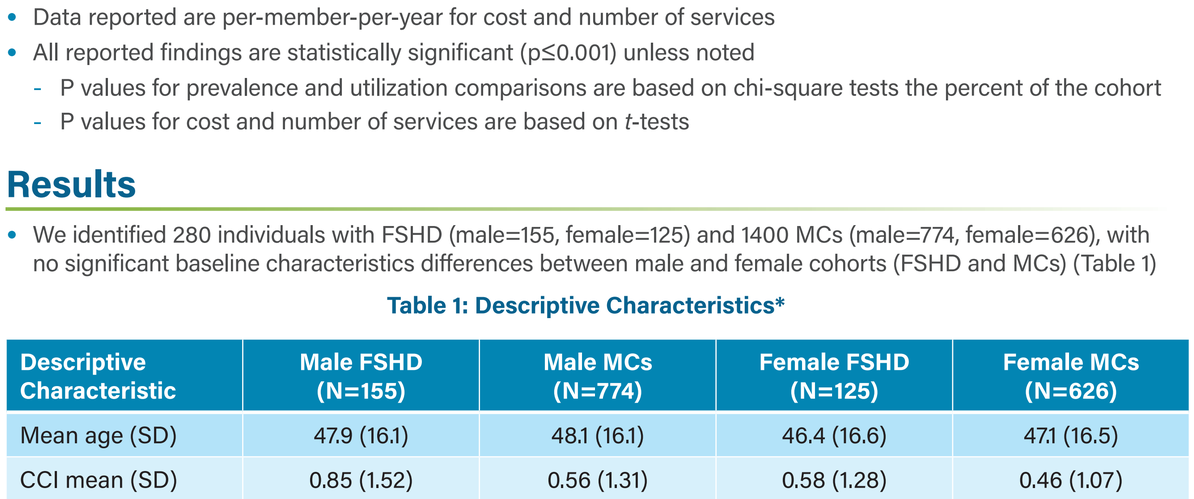
Descriptive Characteristic	Male FSHD (N=155)	Male MCs (N=774)	Female FSHD (N=125)	Female MCs (N=626)
Mean age (SD)	47.9 (16.1)	48.1 (16.1)	46.4 (16.6)	47.1 (16.5)
CCI mean (SD)	0.85 (1.52)	0.56 (1.31)	0.58 (1.28)	0.46 (1.07)
Percent with CCI >1	19.4%	12.9%	12.8%	10.5%

*Descriptive comparisons were not significantly different.

Comorbidities

- There were more comorbid condition specific categories in males than in females (25 vs 17) where prevalence was greater in FSHD vs MCs (Figure 2)
  - Comorbidities more prevalent by relative difference in M-FSHD patients include paralysis, osteoporosis, and adjustment disorders
  - Ear and sense organ disorders, nutritional deficiencies, spondylosis, and circulatory disease were more prevalent in F-FSHD patients by relative difference

Figure 2: Select* Comorbidities by Absolute Prevalence per Cohort



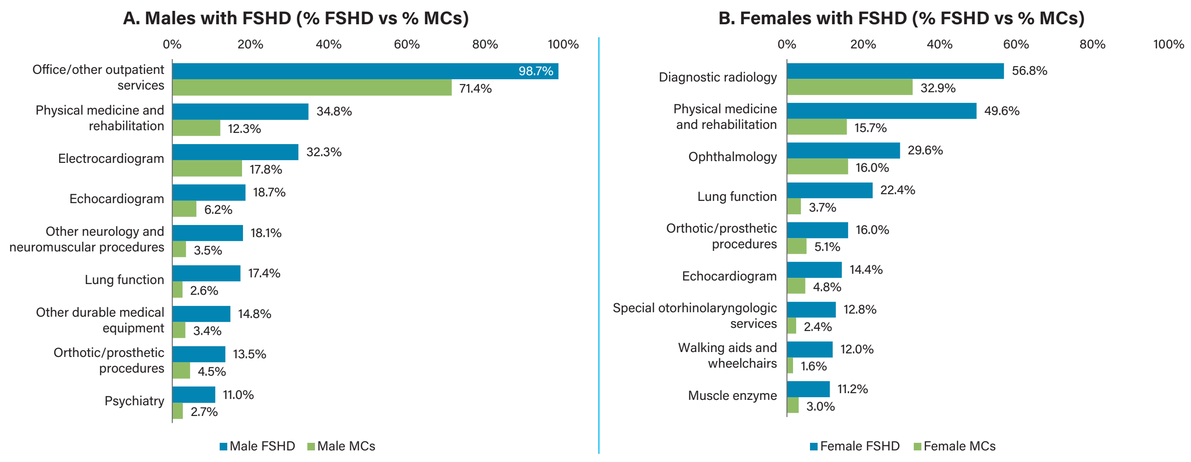
*Comorbidities were selected based on their potential relevance to the FSHD disease course.

Results (continued)

Service Utilization: Procedures

- M-FSHD had 28 procedure categories with greater utilization than MCs out of 158 procedures evaluated (Figure 3)
- F-FSHD had 23 procedure categories with greater utilization than MCs out of 158 procedures evaluated

Figure 3: Select* Procedures with Significant Utilization Differences between Cohorts

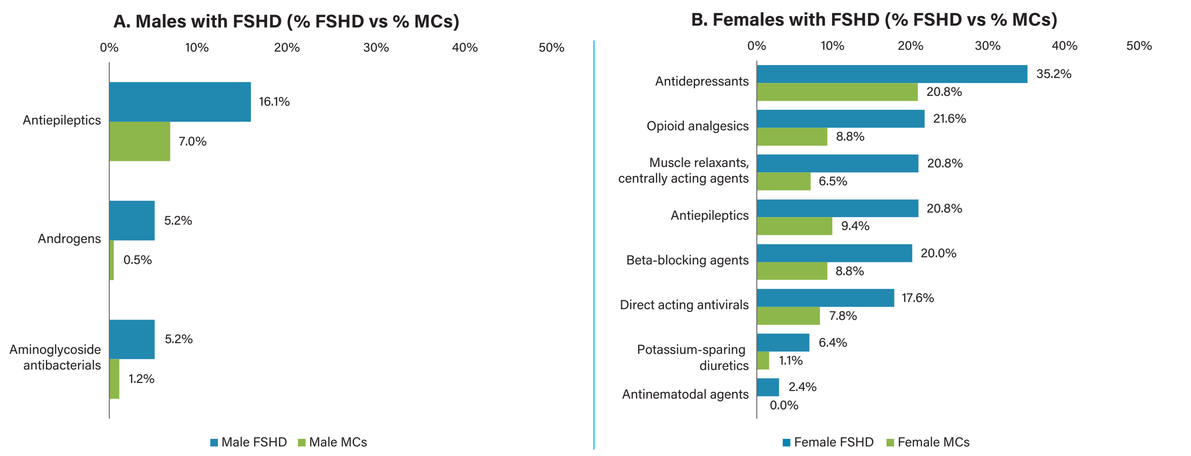


*Procedures were selected based on their potential relevance to the FSHD disease course.

Service Utilization: ATC3 Drugs

- M-FSHD had three ATC3 drug classes with greater prevalence than MCs out of 194 ATC3 drug classes (Figure 4)
- F-FSHD had eight ATC3 drug classes with greater prevalence than MCs out of 194 ATC3 drug classes

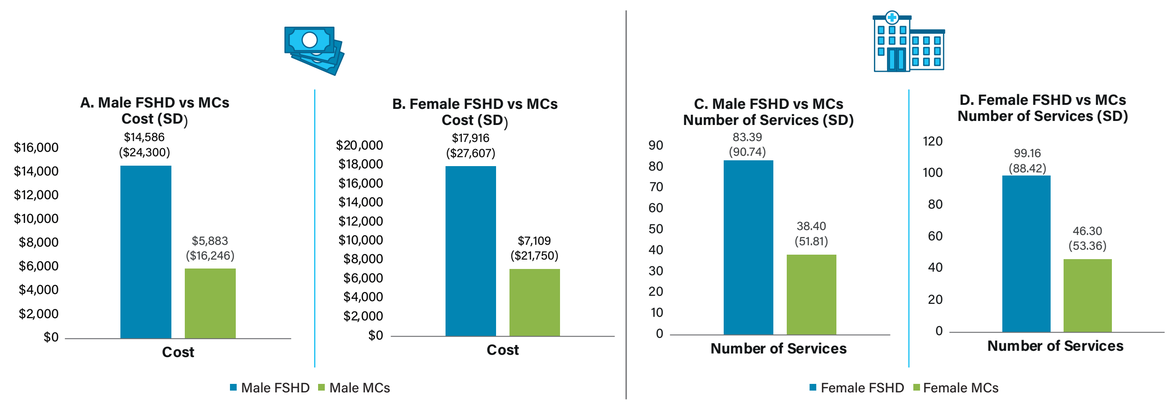
Figure 4: ATC3 Drugs with Significant Utilization Differences between Cohorts



Healthcare Costs

- Compared with MCs, M-FSHD had $8,704 greater healthcare costs, and used 45 more services. Similarly, F-FSHD had $10,807 greater healthcare costs and used 53 more services (Figure 5)

Figure 5: All Medical and Drug Healthcare Costs and Service Utilization



Conclusions

- Compared to their respective MCs, male and female patients with FSHD experienced higher utilization of medical services and prevalence of various comorbidities. In particular, male patients with FSHD had a higher prevalence of osteoporosis than the MCs
- This study suggests variability in the management of FSHD between male and female patients
- There are currently no approved disease-modifying therapies for FSHD. Avidity Biosciences is developing AOC 1020, a first-in-class antibody oligonucleotide conjugate targeting DUX4, the underlying cause of FSHD
  - The US Food and Drug Administration has granted orphan drug status and fast track designation to AOC 1020 for the treatment of FSHD
  - The safety and tolerability of AOC 1020 is currently being evaluated in the Phase 1/2 FORTITUDE™ trial (NCT05747924; 2022-502096-32-00)

References

1. Greco A, et al. *Clin Genet.* 2020;97(6):799–814. 2. Statland JM and Tawil R. *Continuum (Minneap Minn).* 2016;22(6):1916–31. 3. Hamel J, et al. *Neurology.* 2019;93(12):e1180–e1192. 4. Tawil R and Van Der Maarel SM. *Muscle Nerve.* 2006;34(1):1–15. 5. Cohen J, et al. *Trends Mol Med.* 2021;27(2):123–137.

Abbreviations

AHRQ, Agency for Healthcare Research and Quality; AOC, antibody oligonucleotide conjugate; ATC, Anatomical Therapeutic Chemical; CCI, Charlson Comorbidity Index; F-FSHD, female patients with FSHD; FSHD, facioscapulohumeral muscular dystrophy; M-FSHD, male patients with FSHD; MC, matched control; ICD-10, International Classification of Disease Tenth Revision; SD, standard deviation.

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