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

Chamindra Laverty[1], Kathryn A. Munoz[2], Richard A. Brook[3], Nathan L. Kleinman[3], Amy Halseth[2], Hankyung Cho[2], Brad McEvoy[2], Chao-Yin Chen[2], Mark Stahl[2]

[1]UCSD, Rady Children's Hospital, and VA San Diego Healthcare System, [2]Avidity Biosciences, Inc., [3]Better Health Worldwide

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 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.
- 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:

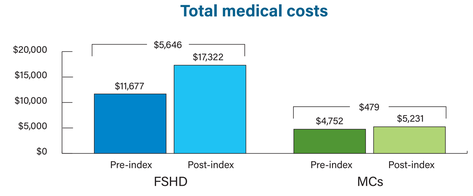
1 FSHD patient	RATIO	5 non-FSHD MCs
≥2 FSHD claims ≥30 days apart		No FSHD
First diagnosis date	INDEX DATE	Assigned by matching process

- 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.

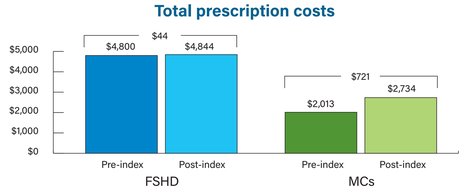
Results

- We identified 79 patients with FSHD and 395 MCs.

Total per member per year medical and prescription costs

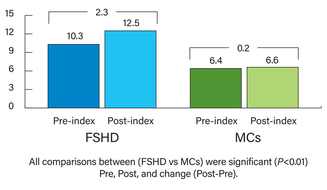


Total medical costs comparisons between the FSHD and MCs cohorts were highly significant ($P<0.0001$) in the pre- and post-periods. Change in total medical costs (Post-Pre) was significant in FSHD patients ($P<0.05$) and not significant in MCs.

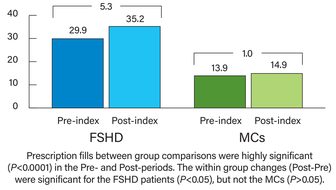


Total prescription costs between group comparisons were significant ($P<0.05$) in the Pre-period. Between group comparisons in the Post-period and the change (Post-Pre) for both cohorts were non significant ($P>0.05$).

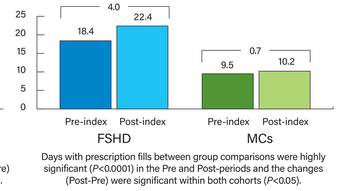
Total per member per year prescription utilization



All comparisons between (FSHD vs MCs) were significant ($P<0.01$) Pre, Post, and change (Post-Pre).



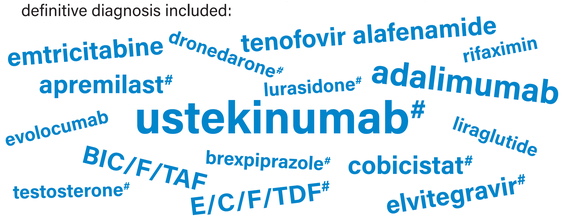
Prescription fills between group comparisons were highly significant ($P<0.0001$) in the Pre- and Post-periods. The within group changes (Post-Pre) were significant for the FSHD patients ($P<0.05$), but not the MCs ($P>0.05$).



Days with prescription fills between group comparisons were highly significant ($P<0.0001$) in the Pre and Post-periods and the changes (Post-Pre) were significant within both cohorts ($P<0.05$).

- 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.

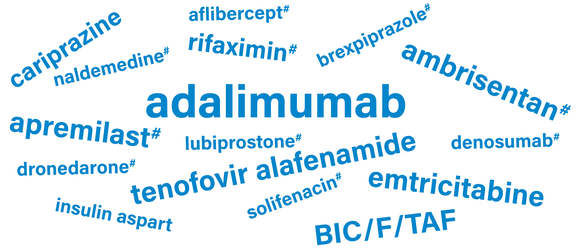
- Specialty pharmacy products used by the FSHD cohort prior to a definitive diagnosis included:



No utilization by MCs.
BIC/F/TAF=emtricitabine, tenofovir alafenamide and bictegravir.[6]
E/C/F/TDF=emtricitabine, tenofovir alafenamide, elvitegravir and cobicistat.[6]
Font size is relative to the cost of the Rx.

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:



No utilization by MCs.
BIC/F/TAF=emtricitabine, tenofovir alafenamide and bictegravir.[6]
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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

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.
6. HIV i-BASE, available at https://i-base.info/guides/passport/drug-names

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