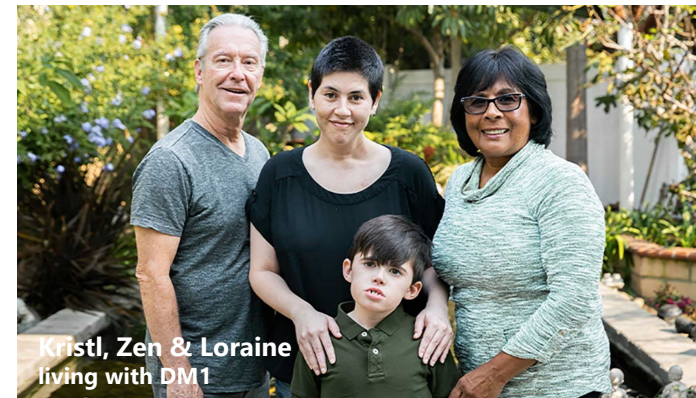




# **Facioscapulohumeral Muscular Dystrophy (FSHD) Mobility Aids and Healthcare Utilization After Diagnosis from a Real-World Data Analysis**

**Elizabeth (Lisa) Ackermann, PhD, Avidity Biosciences, Inc.**

CONFIDENTIAL



**Kristl, Zen & Loraine**  
living with DM1



**Nathan**  
living with DMD



**Josh**  
living with FSHD

## 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\***



## 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

# 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

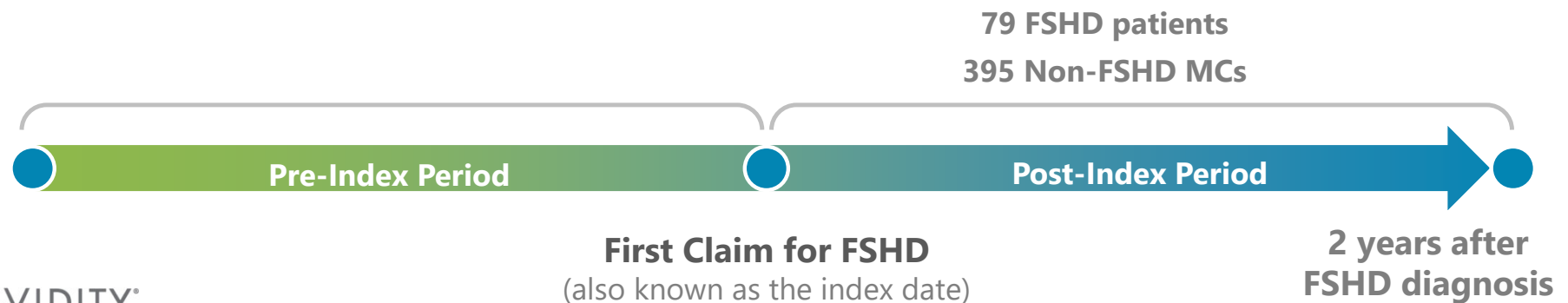
Patients with FSHD were required to have:

- $\geq 2$  claims specifying diagnoses code [ICD-10-CM=G71.02]
- $\geq 30$  days between claims

Index date: first FSHD claim

Continuous Eligibility for 4 years (48 months):

- $\geq 2$  years (24 months) pre-index
- $\geq 2$  years (24 months) post-index




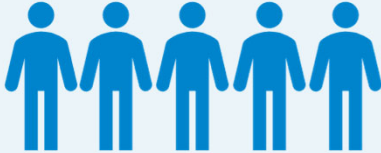
## Patients to “Matched” Controls (MC)

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)

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

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
  - Current Procedural Terminology (CPT) codes

**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

Descriptive Characteristics	FSHD Patients	Non-FSHD MCs
	Mean or Percent	Mean or Percent
Age in years, Mean (Standard Deviation)	47.9 (17.9)	48.4 (17.5)
Age < 18	6.3%	5.3%
18 ≤ Age < 65	81.0%	82.0%
65 ≤ Age	12.7%	12.7%
Female	43.0%	44.3%

### Plan Type

Plan Type	FSHD Patients	Non-FSHD MCs
	Percent	Percent
Preferred Provider Organization	81.0%	79.7%
Health Maintenance Organization	12.7%	12.9%
Point of Service Plan	3.8%	3.8%
Consumer Directed Health Care	2.5%	3.5%

### Payer Type

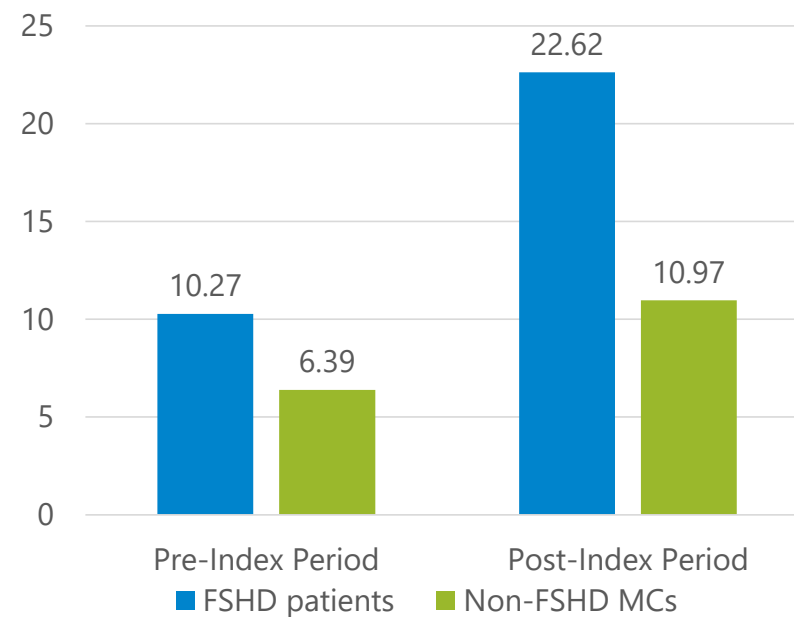
Payer Type	FSHD Patients	Non-FSHD MCs
	Percent	Percent
Commercial	60.8%	63.0%
Self-Insured	31.6%	30.6%
Medicare Advantage	5.1%	3.8%
Medicare Supplemental	2.5%	2.5%

# FSHD Patients Have More Comorbidity Burden Than MCs

Charlson Comorbidity Index

Descriptive Characteristics	FSHD Patients (N=79)		Non-FSHD MCs (N=395)	
	Mean or Percent	Standard Deviation	Mean or Percent	Standard Deviation
Pre-index Charlson Comorbidity Index*	1.13	1.65	0.74	1.67
Percent with Pre-index CCI > 1	27.8%		14.2%	
Post-index Charlson Comorbidity Index*	1.37	2.13	0.82	1.73
Percent with Post-index CCI > 1*	29.1%		17.5%	

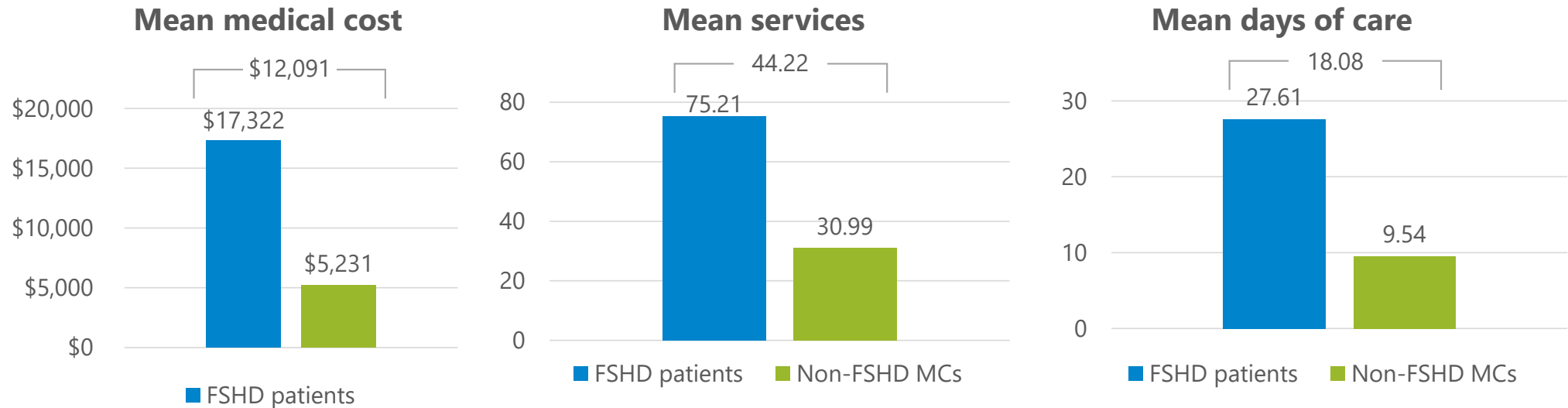
Number of ICD-10 Diagnoses



# 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

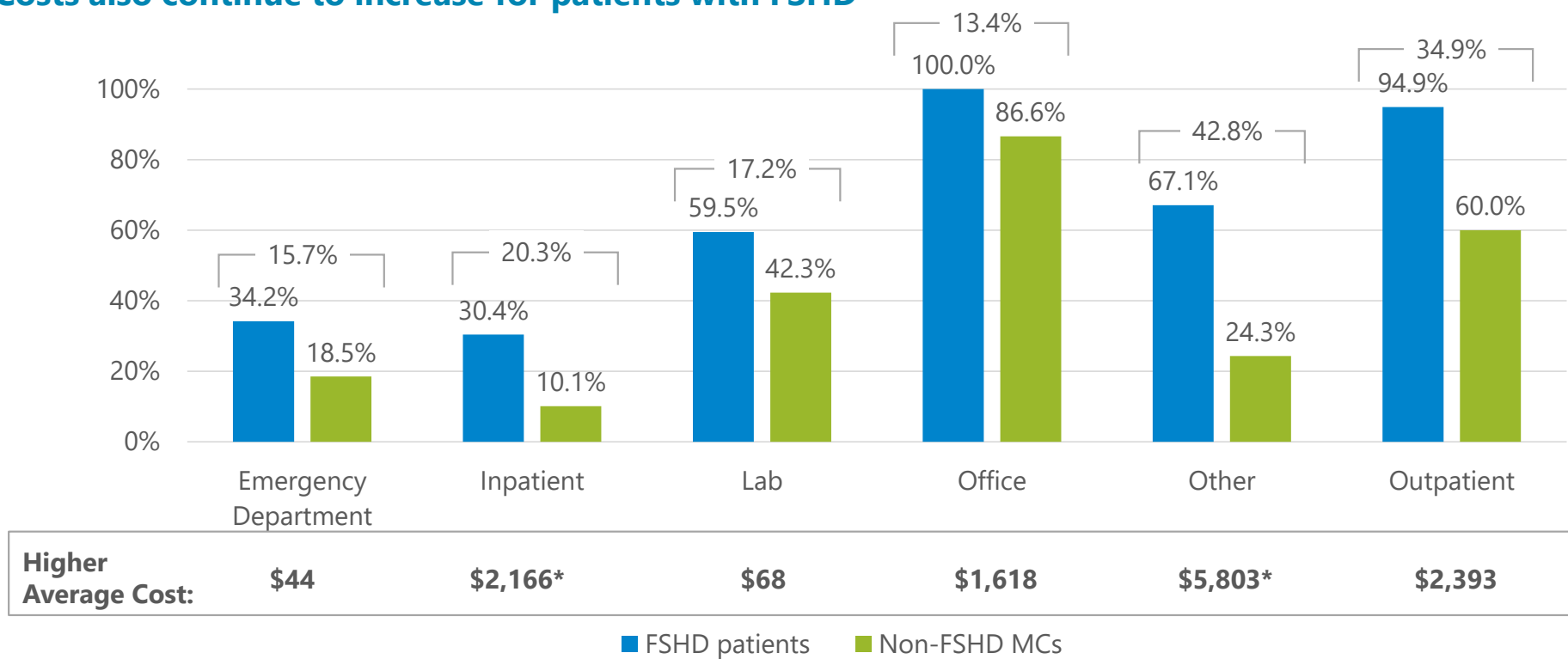
- 44 procedures were significantly more prevalent after a diagnosis



Between cohort comparisons for all metrics,  $P < 0.0001$

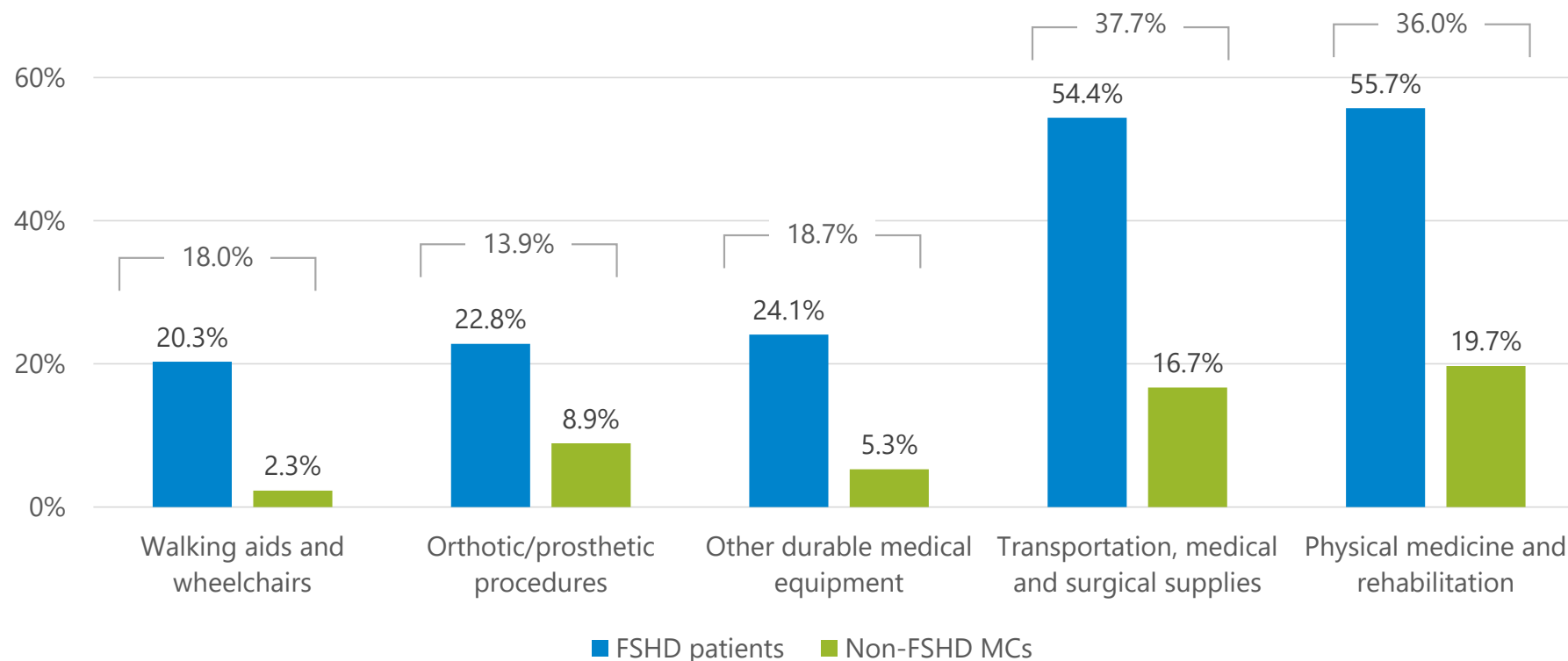
# Healthcare Services After Index Date are Higher in Patients with FSHD

Costs also continue to increase for patients with FSHD



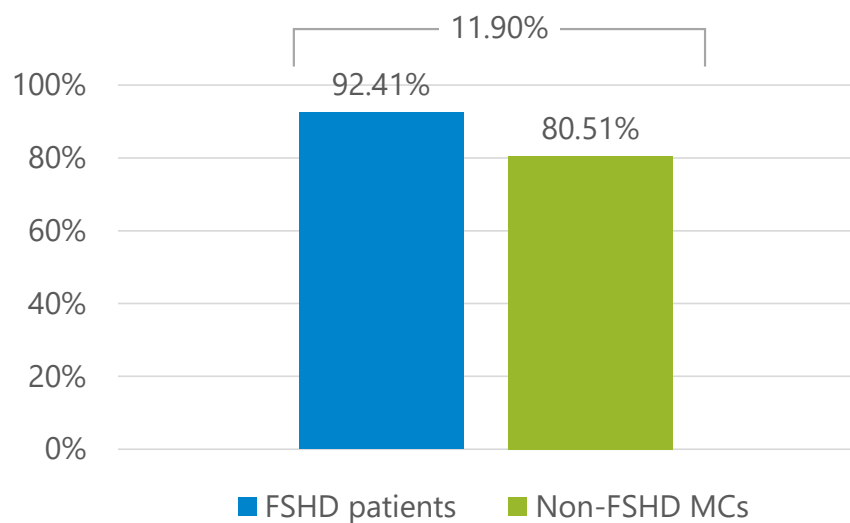
# Patients With FSHD Required More Mobility Related Procedures and Durable Medical Equipment

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

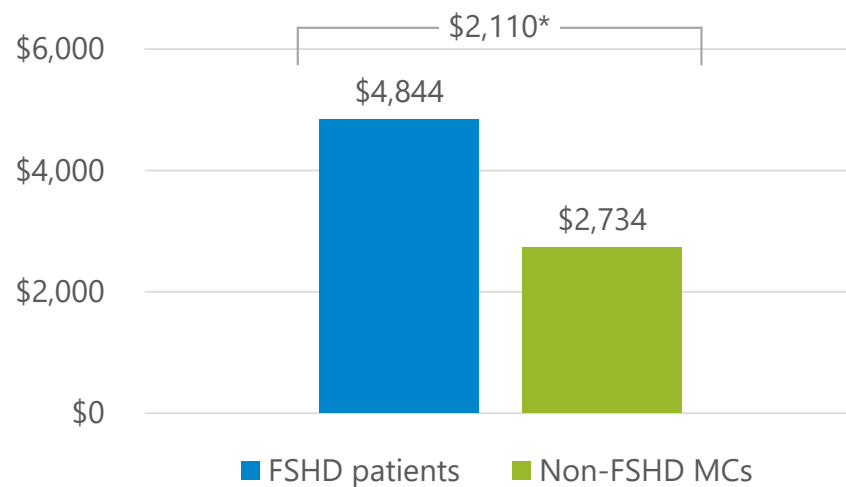


# FSHD Patients Experience Higher Prescription Burdens During the Post-Index Period

Percent of cohort with claims for prescription drugs

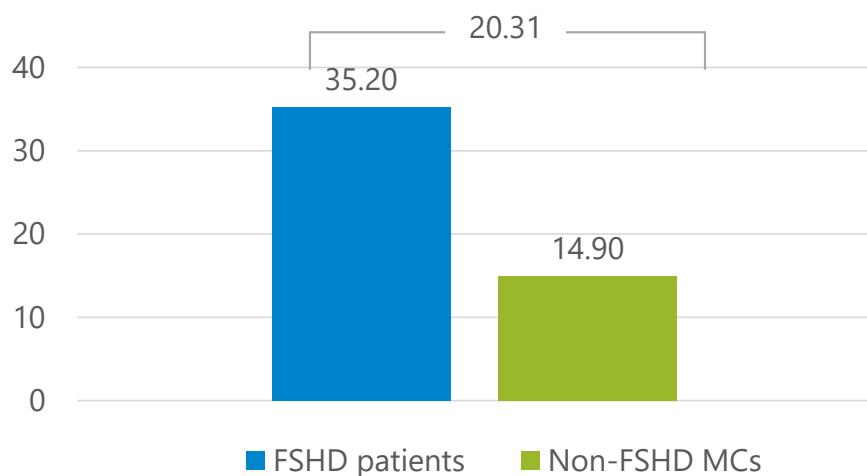


Cost of prescription drugs

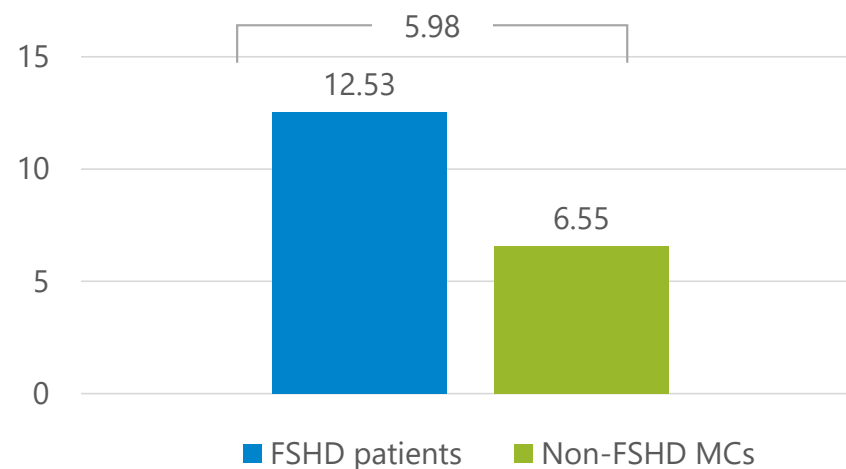


## FSHD Patients Experience Higher Prescription Burdens During the Post-Index Period

Average number of prescription fills

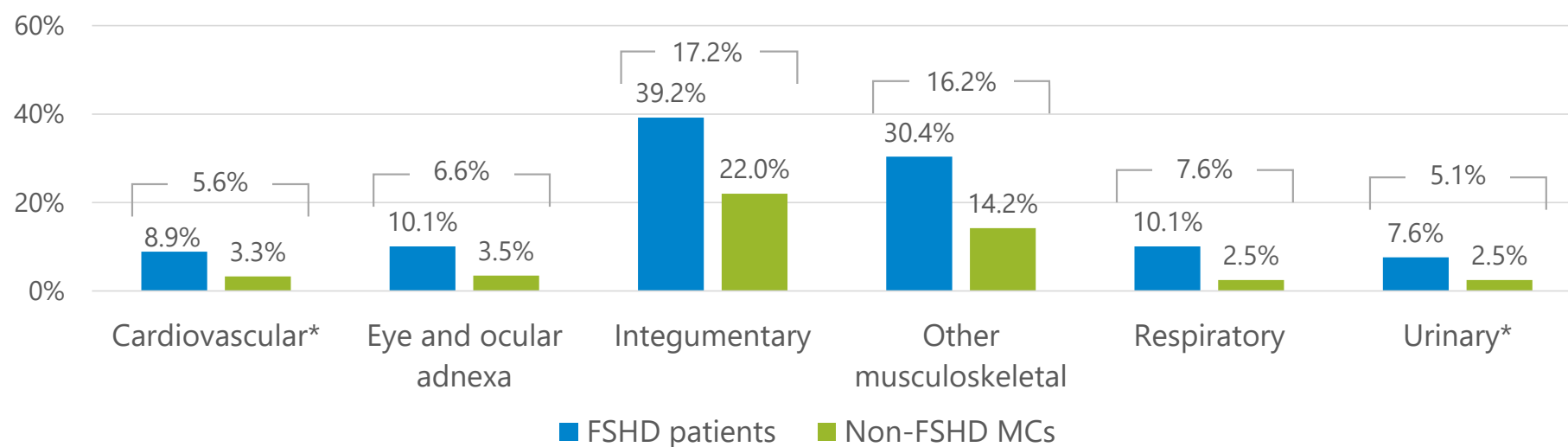


Average number of drugs per patient



# Patients With FSHD Have More Surgical Interventions During the Post-index Period

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

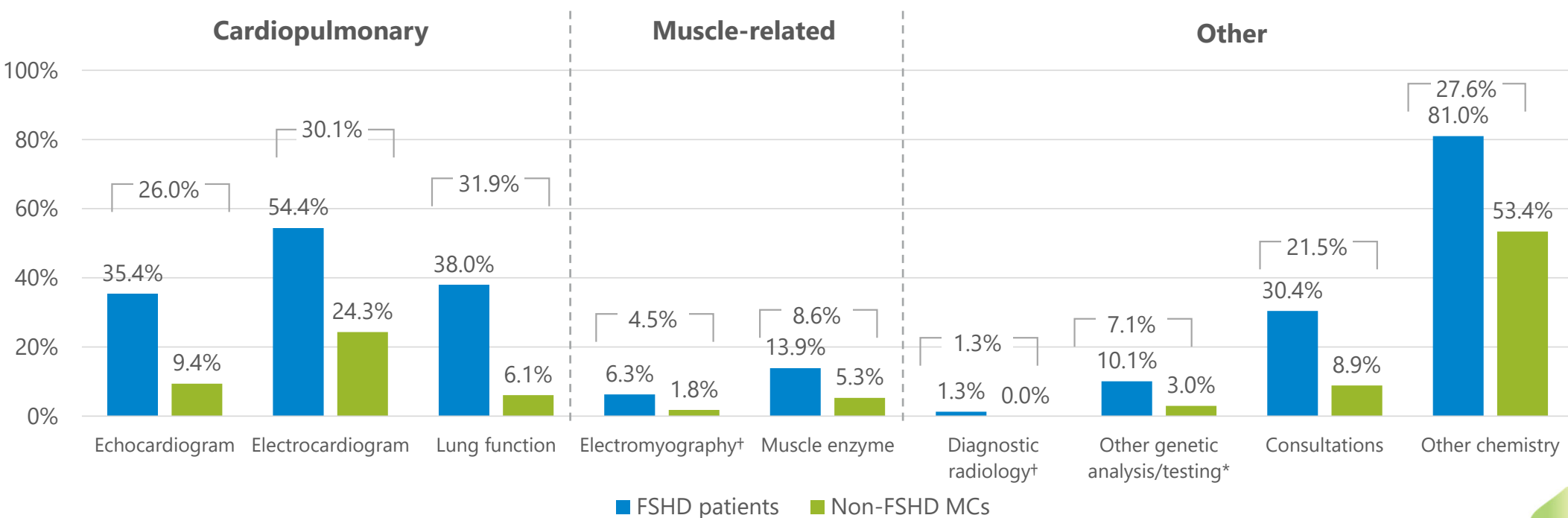


**35.3% types of surgery were more frequently needed by patients with FSHD compared with non-FSHD individuals**



# 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





## 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/](http://www.aviditybiosciences.com/platform/publications/)

CONFIDENTIAL

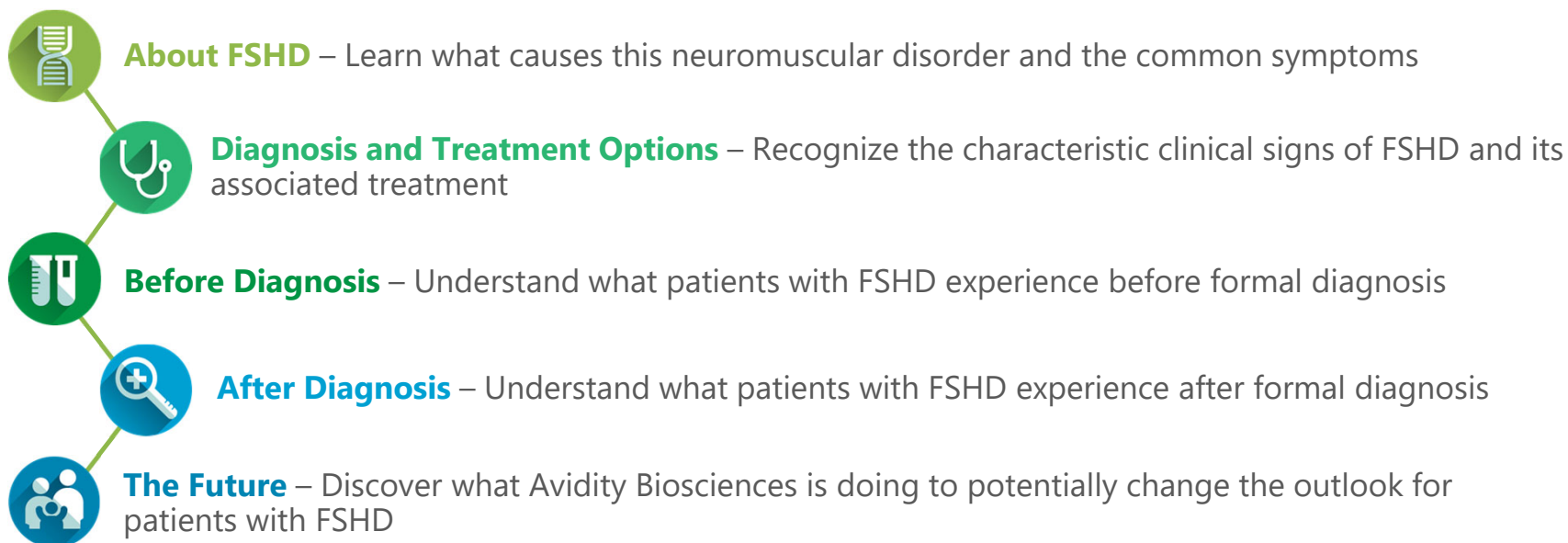


# Backup

CONFIDENTIAL

# The FSHD Patient Journey

The journey begins before diagnosis and continues throughout their lifetime





## An FSHD Patient's Perspective

After diagnosis, healthcare costs continue to significantly increase



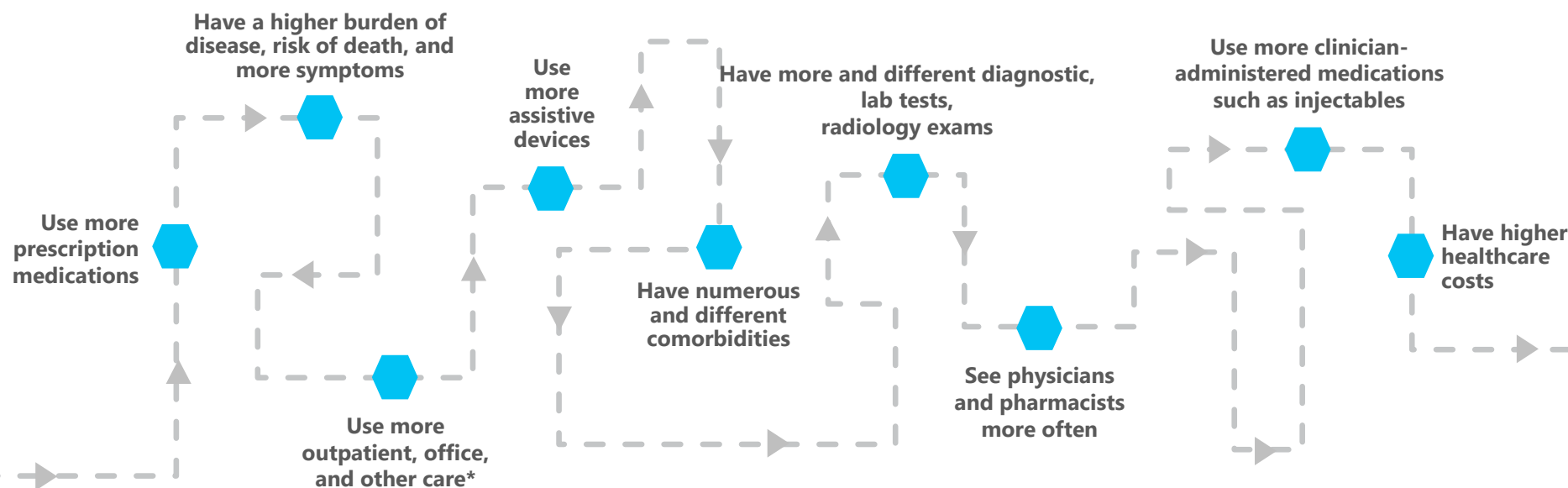
*Medical care you need isn't always covered by insurance. That means **more out-of-pocket expenses for you**, and it only increases as your disability increases.*

— **PATIENT WITH FSHD\***



# After Diagnosis Summary

The burden continues to increase as the disease progresses



# Lung Function and Other Chemistry

## Lung Function

CPT	Description
94010	Breathing capacity test
94011	Up to 2 yrs old, spirometry
94012	= 2 yrs, spirometry w/dilator
94013	= 2 yrs, lung volumes
94014	Patient recoded spirometry
94015	Patient recorded spirometry
94016	Review patient spirometry
94060	Evaluation of wheezing
94070	Evaluation of wheezing
94200	Lung function test (mbc/mvv)
94240	Residual lung capacity
94250	Expired gas collection
94260	Thoracic gas volume
94350	Lung nitrogen washout curve
94360	Measure airflow resistance
94370	Breath airway closing volume
94375	Respiratory flow volume loop
94400	Co2 breathing response curve
94450	Hypoxia response curve

CPT	Description
94680	Exhaled air analysis, o2
94681	Exhaled air analysis, o2/co2
94690	Exhaled air analysis
94720	Monoxide diffusing capacity
94750	Pulmonary compliance study
94770	Exhaled carbon dioxide test
94640	Pressurized or nonpressurized inhalation treatment for acute airway obstruction for therapeutic purposes and/or for diagnostic purposes such as sputum induction with an aerosol generator, nebulizer, metered dose inhaler or intermittent positive pressure breathing (IPPB) device
94726	Spirometry, Lung Volume
94727	Spirometry, Lung Volume
94729	Diffusion Capacity
94150	Spirometry
94728	Lung Volume
94617	Pulmonary stress testing
94618	Pulmonary stress testing
94619	Pulmonary stress testing
94621	Pulmonary stress testing

## 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



# Patients With FSHD Have More Hospital Admissions After Diagnosis

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

