Specialty Pharmacy Products Used by Patients with Myotonic Dystrophy 2-Years Pre- and Post-Diagnosis

John W. Day[1], Kathryn A. Munoz[2], Chao-Yin Chen[2], Richard A. Brook[3], Nathan L. Kleinman[3], Brad McEvoy[2], Hankyung Cho[2], Mark Stahl[2], Li-Jung Tai[2]

[1]Stanford Neuroscience Health Center, [2]Avidity Biosciences, [3]Better Health Worldwide

Background

- Myotonic dystrophy (DM) types 1 and 2 are dominantly inherited multisystem disorders that can cause progressive weakness and myotonia along with variable cardiopulmonary, gastrointestinal, endocrine, and neurological manifestations that adversely affect quality of life.[1-3]
- Literature describing the DM patient journey and specialty pharmacy products used are limited.
- There currently are no approved disease-modifying therapies for DM.[3]

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 DM.

Study Design and Methods

- We used PharMetrics deidentified U.S. claims (Jan 2010—Mar 2021) to retrospectively evaluate care for:

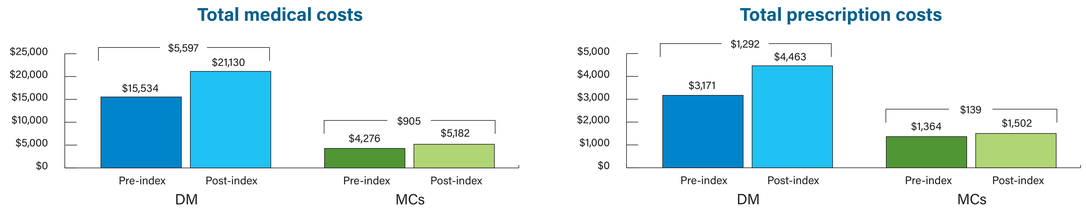
1 DM patient	RATIO	5 non-DM MCs
≥2 DM claims ≥30 days apart		No DM
First diagnosis date	INDEX DATE	Assigned by matching process

- 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 findings are significant (P<0.01) unless noted.

Results

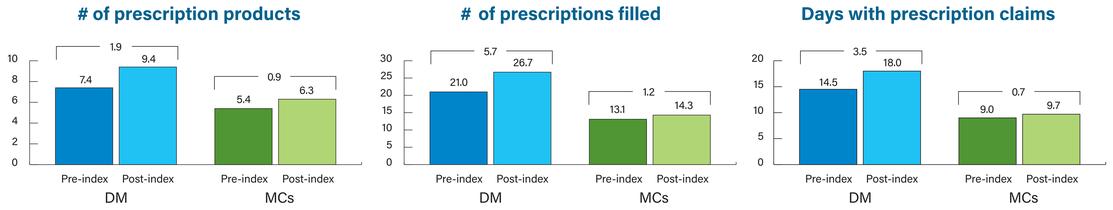
- We identified 519 patients with DM and 2,595 MCs.

Total per member per year medical and prescription costs



All between cohort (DM vs. MCs) comparisons in the Pre-index and Post-index periods significant (P<0.0001).
The changes (Post-Pre) within the DM cohort for total medical costs and the MCs for total prescription costs were not significant.

Total per member per year prescription utilization



All within cohort differences (Post vs. Pre) and between cohort differences (DM vs. MCs) in the Pre-index period, Post-index period, and the (Post-Pre) changes are highly significant (P<0.0001).

- Pre-diagnosis, patients with DM filled specialty pharmacy products for:
  - Pulmonary arterial hypertension and irritable bowel syndrome.
  - Anticoagulants, antifungals, and biologic dyslipidemia medications.
  - Products with FDA indications for cancer, multiple sclerosis, and Crohn's disease.
- Specialty pharmacy products used by the DM cohort prior to a definitive diagnosis included:

None of the medications were used in the MC cohort.
Font size is relative to the cost of the Rx.

Results (continued)

- Post-diagnosis, patients with DM:
  - Continued use of specialty pharmacy products for pulmonary arterial hypertension, dyslipidemia, and products with FDA indications for anticoagulation, multiple sclerosis, and Crohn's disease.
  - Initiated antivirals, anxiolytics, wakefulness-promoting agents, and products with FDA indications for eczema, myelofibrosis, hepatitis C, and ocular disorders.
- Post-index specialty pharmacy drugs used by the DM cohort included:

Used by both cohorts. *Utilization significantly different P<0.0001.
Font size is relative to the cost of the Rx.

Conclusions

- DM patients' healthcare utilization and costs are higher than MCs pre- and post-diagnosis.
- DM patients filled more overall prescriptions and specialty pharmacy products for symptomatic management, illustrating the complexities of DM diagnosis and the burden of DM to the patients and society.
- Approved disease modifying therapies that treat DM may reduce the need for symptomatic management.

References

1. Udd B and Krahe R. *Lancet Neurol.* 2012;11(10):891–905.
2. Gourdon G and Meola G. *Front Cell Neurosci.* 2017;11:101.
3. LoRusso S, *et al. Neurotherapeutics.* 2018;15(4):872–84.