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

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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
- Literature describing the DM patient journey and specialty pharmacy products used are limited.
- There currently are no approved disease-modifying therapies for DM.2

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:

<table>
<thead>
<tr>
<th>1 DM patient</th>
<th>5 non-DM MCs</th>
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</thead>
<tbody>
<tr>
<td>≥2 DM claims</td>
<td>≥30 days apart</td>
</tr>
<tr>
<td>No DM</td>
<td>Assigned by matching process</td>
</tr>
<tr>
<td>First diagnosis date</td>
<td>INDEX DATE</td>
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- Cohorts were matched on index month, baseline age, 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<0.001) unless noted.

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

### Total per member per year medical and prescription costs

![Graph showing total per member per year medical and prescription costs.]

<table>
<thead>
<tr>
<th></th>
<th>Pre-index</th>
<th>Post-index</th>
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</thead>
<tbody>
<tr>
<td>Total medical costs</td>
<td>Pre-index</td>
<td>Post-index</td>
</tr>
<tr>
<td>DM</td>
<td>$12,000</td>
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<tr>
<td>MCs</td>
<td>$15,000</td>
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### Total prescription costs

![Graph showing total prescription costs.]

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- 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:
  - Riociguat
  - Ustekinumab
  - Dimethyl fumarate
  - Bosentan
  - Temozolomide

- 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:

### 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