Prevalence of Healthcare Conditions and Services Used by Patients with Myotonic Dystrophy (DM) Pre- and Post-Diagnosis: a Real-World Data Analysis

John Day, MD, PhD; Kathryn A. Munoz, PhD, MPH; Richard A. Brook, MS, MBA; Bradley McEvoy, DrPH; Kelly DiTrapani, BA, BSN; Nathan L. Kleinman, PhD; Chao-Yin Chen, PhD; Mark C. Stahl, MD, PhD; Li-Jung Tai, MD, PhD

1Stanford University Medical Center; Avidity Biosciences, Inc.; Better Health Worldwide. All authors have met authorship criteria.

Data previously presented at the 2022 Muscular Dystrophy Association Clinical & Scientific Conference.

Objectives

Describe the changes in outcomes for healthcare conditions, services, costs, and care days for patients with DM compared with matched controls (MCs) 2 years post-diagnosis versus 2 years pre-diagnosis.

Background

• DM is a rare, dominantly inherited, monogenic, multisystem disease that causes myotonia, progressive muscle weakness, and atrophy, along with respiratory, gastrointestinal, cardiac, and central nervous system dysfunction, which significantly impacts quality of life.

• There are two major types of DM [type 1 and type 2]1

• Patients experience significant physical limitations, pain, fatigue, and a negative impact on wellbeing3

• Currently there are no approved therapeutics for DM, and there remains high unmet need for disease-modifying therapies3

• Real-world data characterizing the patients’ pre- and post-diagnosis changes are limited

Methods

• Retrospective database analysis to compare outcomes for patients with DM versus MCs

  - Database: IQVIA US PharMetrics® Plus
  - Timeframe: January 2010 through March 2021

• The DM cohort is defined as having ≥2 DM claims ≥30 days apart

• Claims identified by International Classification of Disease Ninth Revision (ICD-9) code 359.21 or Tenth Revision (ICD-10) code G71.1, which does not differentiate between DM subtypes

• The first diagnosis date was used for the index date

• DM patients were matched to a 5% random sample of eligible non-DM controls

  - Matching was done using 8 Matchit procedure, with nearest neighbor matching (exact matching on month of index date)

• Cohorts were matched (5:1:1:1:1) on index month and baseline age, region, gender, plan, and payers types

• The index date was the beginning of the post-index evaluation period

• All subjects (patients and MCs) were required to have a minimum of 48 months of continuous data:

  - 24 months prior to their index date

  - 24 months following [post] their index date (includes the index date)

• Changes in outcomes were measured as 2 years post-diagnosis minus 2 years pre-diagnosis using:

  - Location of care for overall care

  - 283 US Agency for Healthcare Research and Quality (AHRQ) condition categories

• Post-diare changes were compared within cohorts (using McNemar tests) and between cohorts (using t-tests)

• All presented comparisons were significant (p<0.0001 unless noted)

• Based on the 283 comparisons, those p-values ≤0.000177 (0.05/283) are considered highly significant

Results

We identified 519 DM patients and 2,595 MCs

• Descriptive characteristics were similar between cohorts (Table 1)

• The cohorts had significant (p<0.0001) differences for the Charlson Comorbidity Index (Table 2)

• The cohorts had changes [Post-Pre] in costs and days of service by location of care (Table 3)

• DM patients:

  - AHRQ prevalence changed significantly in 58 categories (*p<0.0001; #p<0.01)

  - The index date was the beginning of the post-index evaluation period

  - AHRQ prevalence changed significantly in 58 categories (as described in Table 2)

Table 1: Age, US Region, Insurance, and Payor Types Were Similar Between DM Patients and MCs

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>DM Patients (N=519)</th>
<th>MCs (N=2,595)</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, mean (SD) years</td>
<td>47.6% (12.4)</td>
<td>47.6% (12.4)</td>
<td>0.87</td>
</tr>
<tr>
<td>Age, mean (SD) years</td>
<td>47.6% (12.4)</td>
<td>47.6% (12.4)</td>
<td>0.87</td>
</tr>
<tr>
<td>Age, total</td>
<td>11.0%</td>
<td>11.0%</td>
<td>0.49</td>
</tr>
<tr>
<td>Age, total</td>
<td>11.0%</td>
<td>11.0%</td>
<td>0.49</td>
</tr>
<tr>
<td>Region US</td>
<td>20.8%</td>
<td>20.8%</td>
<td>0.51</td>
</tr>
<tr>
<td>Region US</td>
<td>20.8%</td>
<td>20.8%</td>
<td>0.51</td>
</tr>
<tr>
<td>Gender</td>
<td>26.7%</td>
<td>26.7%</td>
<td>0.56</td>
</tr>
<tr>
<td>Gender</td>
<td>26.7%</td>
<td>26.7%</td>
<td>0.56</td>
</tr>
<tr>
<td>Insurance type</td>
<td>35.3%</td>
<td>35.3%</td>
<td>0.47</td>
</tr>
<tr>
<td>Insurance type</td>
<td>35.3%</td>
<td>35.3%</td>
<td>0.47</td>
</tr>
<tr>
<td>Payor type</td>
<td>4.6%</td>
<td>4.6%</td>
<td>0.99</td>
</tr>
<tr>
<td>Payor type</td>
<td>4.6%</td>
<td>4.6%</td>
<td>0.99</td>
</tr>
<tr>
<td>Source</td>
<td>6.7%</td>
<td>6.7%</td>
<td>0.99</td>
</tr>
<tr>
<td>Source</td>
<td>6.7%</td>
<td>6.7%</td>
<td>0.99</td>
</tr>
<tr>
<td>specialty</td>
<td>5.4%</td>
<td>5.4%</td>
<td>0.11</td>
</tr>
<tr>
<td>specialty</td>
<td>5.4%</td>
<td>5.4%</td>
<td>0.11</td>
</tr>
</tbody>
</table>

Table 2: Before and After Diagnosis, Charlson Comorbidity Index Scores Were Higher for DM Patients Versus MCs

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>DM Patients (N=519)</th>
<th>Matched MCs (N=2,595)</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Charlson score</td>
<td>1.08 (1.83)</td>
<td>0.57 (1.30)</td>
<td>0.0001</td>
</tr>
<tr>
<td>Charlson score</td>
<td>1.08 (1.83)</td>
<td>0.57 (1.30)</td>
<td>0.0001</td>
</tr>
</tbody>
</table>

Table 3: Costs of and Days of Service (Before, After, and Changes (Post minus Pre)) Were Higher for DM Patients for “Emergency Department”, “Inpatient”, and “All Locations of Care”

<table>
<thead>
<tr>
<th>Location</th>
<th>DM Patients (SD)</th>
<th>Matched MCs (SD)</th>
<th>Difference</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Emergency department</td>
<td>$505 ($317)</td>
<td>$268 ($175)</td>
<td>$237*</td>
<td>0.0003</td>
</tr>
<tr>
<td>Emergency department</td>
<td>$505 ($317)</td>
<td>$268 ($175)</td>
<td>$237*</td>
<td>0.0003</td>
</tr>
</tbody>
</table>

Conclusions

• Healthcare utilization increased significantly in DM patients following diagnosis and was higher both overall and in different AHRQ categories than in MCs

• This likely reflects the need to investigate and manage previously unsuspected manifestations of DM following formal diagnosis

• Future research should confirm if these findings hold true in longer-term follow-up

• These data highlight the burden of disease for DM patients, including higher costs, more days of care, and more prevalent and costly comorbidity management and the need for therapeutic interventions

• Based on the high unmet need, Avidity Biosciences is investigating AOC 1001 for the potential treatment of myotonic dystrophy type 1

Figure 1: DM Patients Had Higher Increases in Prevalence For AHRQ Categories of “Other Nervous System Disorders”, “Cardiac Dysrhythmias”, and “Other Lower Respiratory Disease” Versus MCs

Figure 2: DM Patients Had Higher Increases in Costs in AHRQ Categories of “Other Nervous System Disorders”, “Unclassified Codes”, “Other Fractures”, and “Developmental Disorders” Versus MCs

Figure 3: DM Patients Had Higher Increases in “Other Nervous System Disorders” and “Respiratory-Related” Services Versus MCs

Abbreviations:

AHRQ, US Agency for Healthcare Research and Quality; DM, myotonic dystrophy; MC, matched control; PMPY, per-member per-year; SD, standard deviation.

References:


*All comparisons highly significant (p<0.0001)

This study was supported by Avidity Biosciences, Inc. and grants from the Muscular Dystrophy Association and the National Institute of Neurological Disorders and Stroke (1R01NS110127-01A1). WMS P.209

Appendix A: Table of Sensitivity Analyses for Categories of Service

Figure 1: DM Patients Had Higher Increases in Prevalence For AHRQ Categories of “Other Nervous System Disorders”, “Cardiac Dysrhythmias”, and “Other Lower Respiratory Disease” Versus MCs

Figure 2: DM Patients Had Higher Increases in Costs in AHRQ Categories of “Other Nervous System Disorders”, “Unclassified Codes”, “Other Fractures”, and “Developmental Disorders” Versus MCs

Figure 3: DM Patients Had Higher Increases in “Other Nervous System Disorders” and “Respiratory-Related” Services Versus MCs


© Avidity Biosciences, Inc. 2019. Partial Clinical Hold on New Participant Enrollment in Phase 1/2 MARINA™ Trial. (2022-09)