INTRODUCTION

Duchenne muscular dystrophy (DMD) is a rare, X-linked, severely debilitating, and ultimately fatal neuromuscular disease characterized by progressive muscle weakness. Eteplirsen is approved in the US treat the DMD patients with who have confirmed mutation of the DMD gene amenable to exon 51 skipping. The rarity of DMD makes it difficult to assess real-world outcomes in these patients. This study uses a data set with claims covering a majority of the US population and linked electronic medical records (EMR) to assess DMD-related health and resource utilization outcomes in treated and untreated DMD patients.

OBJECTIVE

The objective of this study was to provide real-world perspective on healthcare resource utilization and significant disease progression events in patients with DMD treated with eteplirsen and those treated with standard care alone.

METHODS

DATA AND MATCHING

- Linked administrative claims and EMR data (2011-2020) from Decision Resources Group data repository were used.
- Patients with both administrative claims and EMR data in whom SNOMED-CT codes could identify disease were selected.
- Eteplirsen-treated and standard of care cohorts were compared for post-baseline utilization and significant disease progression events in patients with DMD treated with eteplirsen and those treated with standard care alone.

PATIENT CHARACTERISTICS

- Eteplirsen-treated and standard of care patients were matched on age, disease stage, and key health events using a two-stage matching algorithm.
  - Stage 1 (exact matching): each treated patient was matched exactly with the full set of untreated patients on age at index and disease stage at index.
  - Stage 2 (propensity score matching): within the exact matching sets of treated and untreated patients, patients were further matched based on DMD-related health events in the baseline period using propensity score.

REGRESSION ANALYSIS

- Eteplirsen-treated and standard of care cohorts were compared for post-baseline outcomes using Poisson regression methods, adjusted for length of observation in baseline and follow-up periods, and controlling for age, health stage, and baseline level of each health event measure.
  - Hospitalization days and intensity-adjusted hospital encounters
  - Emergency room (ER) visits and intensity-adjusted emergency room visits
  - Intensive care unit (ICU) days and intensity-adjusted ICU
  - Pulmonary management
  - Cardiac management
  - Tracheostomy
  - Assisted ventilation

RESULTS

SAMPLE SELECTION

<table>
<thead>
<tr>
<th>Total Number of Patients</th>
<th>Eteplirsen-treated</th>
<th>Unmatched</th>
</tr>
</thead>
<tbody>
<tr>
<td>With at least 1 medical claim</td>
<td>480</td>
<td>907</td>
</tr>
<tr>
<td>With available health status</td>
<td>480</td>
<td>955</td>
</tr>
<tr>
<td>At least one-to-one matching (&gt;6 months&lt;sup&gt;‡&lt;/sup&gt;)</td>
<td>333</td>
<td>333</td>
</tr>
<tr>
<td>At least one-to-one matching (&gt;3 months&lt;sup&gt;‡&lt;/sup&gt;)</td>
<td>324</td>
<td>324</td>
</tr>
<tr>
<td>At least one-to-one matching (&gt;0 months&lt;sup&gt;‡&lt;/sup&gt;)</td>
<td>301</td>
<td>301</td>
</tr>
</tbody>
</table>

PATIENT CHARACTERISTICS

- health and resource utilization outcomes in treated and untreated DMD patients.
- A majority of the US population and linked electronic medical records (EMR) to assess DMD-related health and resource utilization outcomes in treated and untreated DMD patients.
- The objective of this study was to provide real-world perspective on healthcare resource utilization and significant disease progression events in patients with DMD treated with eteplirsen and those treated with standard care alone.
- The matching process and Poisson regression also include baseline rates of motorized wheelchair and cough assist device as well as ever have had a motorized wheelchair or cough assist device.
- Baseline yearly average rates are based on Poisson model estimates.
- Secondary endpoints include baseline yearly average rates and adjusted annual rate differences (ARDs) between eteplirsen-treated and untreated patients.
- The primary analysis (>6 months follow-up) demonstrated that Eteplirsen treatment is associated with significantly lower rates of:
  - Adjusted hospital encounter (P=0.007) and hospital days (P=0.005)
  - Adjusted ER (P=0.004) and ER days (P=0.024)
  - Adjusted ICU rates (P=0.038) and days (P=0.038)
  - Pulmonary management (P=0.004)
  - Cardiac management (P=0.001)
  - Tracheostomy (P=0.0011)
  - Assisted ventilation (P<0.01)
- The results in response are statistically significant.

CONCLUSION

This study provides the first real-world evidence of eteplirsen treatment effects on key health outcomes in DMD patients.

ACKNOWLEDGEMENTS AND DISCLOSURES

This study was funded by Sarepta Therapeutics, Inc; J. Iff and C. Gerrits are employees of Sarepta Therapeutics Inc. and may own stock/options in the company; E. Tuttle, D. Gupta and Y. Zhong are employed by Analysis Group, Inc. and received funding from Sarepta Therapeutics Inc. for conducting the interim data analysis and writing support.

REFERENCES

2. Patient characteristics and outcomes for DMD-related health events in the treated and untreated patient populations were compared using Poisson regression methods, adjusted for length of observation in the baseline period using propensity score.
3. Matched sample for DMD-related health events in the baseline period using propensity score.

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