Deflazacort and Prednisone Treatment for Duchenne Muscular Dystrophy (DMD): Real-world Outcomes at Cincinnati Children’s Hospital Medical Center (CCHMC)

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1. Introduction
- Corticosteroids are the standard of care for the treatment of Duchenne muscular dystrophy (DMD), and have modified the natural history of the disease by slowing the progression of motor and pulmonary functional decline and extending survival.
- Deflazacort and prednisone/prednisolone are glucocorticoids that are used in the treatment of DMD.
- Protracted glucocorticoid steroid treatment is associated with linear growth failure, excessive weight gain, and heightened risk of osteoporosis.

2. Objectives
- To assess real-world ambulatory, pulmonary, cardiac, growth, and bone-health outcomes among patients with DMD who received deflazacort or prednisone as part of coordinated, interdisciplinary care at CCHMC.

3. Patients
- Genetically confirmed diagnosis of DMD.
- Initial treatment with either deflazacort or prednisone.
- Patients were categorized into prednisone- and deflazacort-initiated groups based on their first recorded steroid type.
- Loss of ambulation (LoA) was identified as Functional Mobility Scale (FMS) score >4, indicative of patients’ primary use of a wheelchair.
- Kaplan-Meier curves and adjusted Cox proportional hazards analyses were used to assess outcomes by steroid type.

4. Methods
- Outcomes of interest included age at loss of ambulation, age at scoliosis onset, and duration of ambulation.
- Sample sizes were based on assessment frequency of each clinical measure. Patients were required to have an FMS score of ≤4 at their first clinic visit; patients were required to be free of scoliosis at their first clinic visit; patients were required to be free of scoliosis at their first clinic visit.
- Kaplan-Meier curves and adjusted Cox proportional hazards analyses were used to assess outcomes by steroid type; sensitivity analyses were conducted for patients who switched steroid type.

5. Results

5. Results (continued)
- These differences persisted in multivariable models adjusting for age at steroid initiation and year of steroid initiation (both P<0.05) (Table 1).

Table 1: Hazard ratios for ages at loss of ambulation and scoliosis onset

<table>
<thead>
<tr>
<th>Outcome</th>
<th>Unadjusted HR (95% CI)</th>
<th>P-value</th>
<th>Adjusted1 HR (95% CI)</th>
<th>P-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Loss of ambulation</td>
<td>0.55 (0.38, 0.83)</td>
<td>&lt;0.01*</td>
<td>0.45 (0.29, 0.69)</td>
<td>&lt;0.001*</td>
</tr>
<tr>
<td>Scoliosis onset</td>
<td>0.54 (0.29, 1.01)</td>
<td>0.05</td>
<td>0.52 (0.28, 0.97)</td>
<td>&lt;0.05*</td>
</tr>
</tbody>
</table>

6. Limitations
- As a non-randomized comparison of real-world treatment groups, differences in outcomes could be confounded by factors that differ between these groups, such as socio-economic status or care plan adherence.
- Doing data were not available to characterize steroid exposure beyond daily vs. non-daily.

7. Conclusions
This real-world, non-randomized study adds to the evidence from observational studies1 suggesting use of deflazacort with greater preservation of motor and pulmonary function relative to the use of prednisone in DMD, and suggests concurrent preservation of lean body mass and delay of scoliosis.

References

Disclosures
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