

Corticosteroid Use Among Patients with Duchenne Muscular Dystrophy: A 20-Year Population-Based Study

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BACKGROUND

- The progression of Duchenne muscular dystrophy (DMD) is characterized by loss of ambulation (LOA) and upper limb function, cardiorespiratory impairment, and early mortality.¹
- Early and regular use of corticosteroids can prolong the time to key clinical milestones such as LOA.² These are now standard of care for managing DMD in Canada and worldwide.³
- While data on corticosteroid treatment outcomes are available from numerous clinical cohorts and registries,⁴⁻⁷ few data describe corticosteroid use in real-world populations with DMD.^{8,9}

OBJECTIVE

- This study aimed to characterize real-world corticosteroid use among a closed Canadian DMD population across a period spanning the introduction of corticosteroids for the management for DMD.

METHODS

- Linked longitudinal population-based single-payer physician, hospitalization, and outpatient prescription dispensation records from the Manitoba Population Research Data Repository¹⁰ were used to identify all males diagnosed with muscular dystrophy (MD) at ≤10 years of age.
 - Eligible individuals had to initiate corticosteroid treatment during the study period (1998-2020).
 - Eligible individuals were enrolled on their index date, defined as their first healthcare encounter coded with an MD-specific diagnosis during the study period.
- Patient demographics, the distribution of corticosteroid use by type, the proportion of days covered* (PDC), and total duration of coverage were summarized.
- Results were stratified according to birth year to explore differences in treatment in relation to the release of updated guidelines.
 - Patients were stratified into those born earlier than 2005 ('pre-corticosteroid era') vs. during or after 2005 ('post-corticosteroid era').
 - Using 2005 as the delineation allowed approximately 5 years from birth to likely time to initiate corticosteroid use.
- Age at first corticosteroid use was estimated for a subset of patients
 - This subset was an incident cohort with no healthcare resource use related to MD in the two years prior to their index date.

*A measure of adherence reflecting the proportion of days an individual has access to medication

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RESULTS

- Fifty-one patients with DMD were identified (Figure 1). The mean (standard deviation, SD) age at index was 6.3 (5.8) years, and median (interquartile range, IQR) follow up was 11.6 (10.4) years.
- Most (73%) patients were treated with prednisone, 22% with deflazacort, and 5% used both.
- Over the period, the mean (SD) PDC was 0.3 (0.4), and total mean (SD) duration of corticosteroid use per patient was 13.4 (19.6) months (Figure 2).
 - Mean (SD) PDC was lower (0.2 [0.2]) for patients born before 2005 vs. after 2005 (0.6 [0.5]).
 - The duration (IQR) of corticosteroid use was lower (11.2 [21.1]) months) for those born before 2005, vs. after 2005 (17.3 [16.3] months).
- The 16 incident patients from the pre-corticosteroid era were first dispensed corticosteroids at a median (IQR) age of 9 (8), vs. 6 (4) years of age among the 15 incident patients from the post-corticosteroid era.

Figure 1: Study cohort diagram

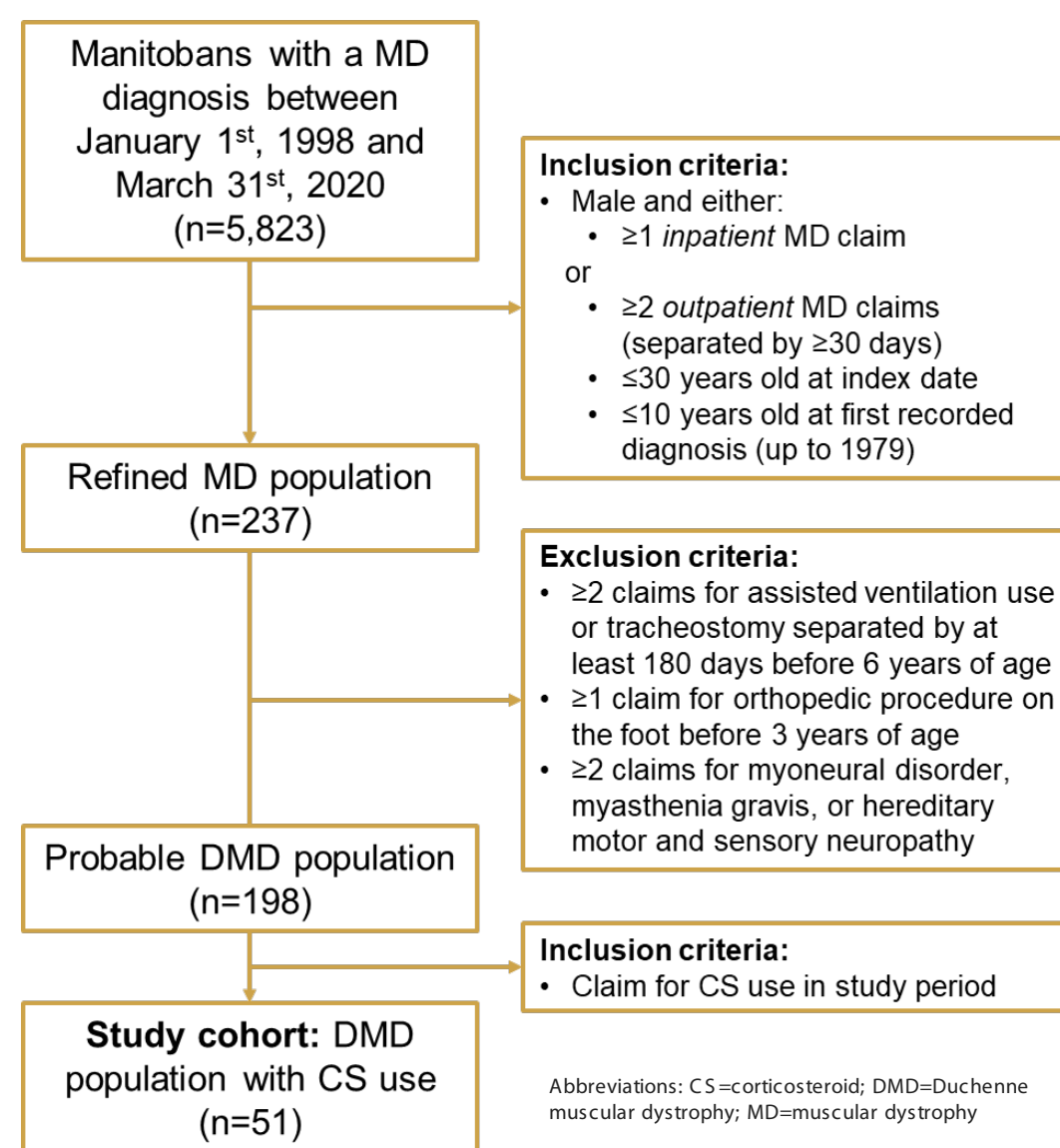
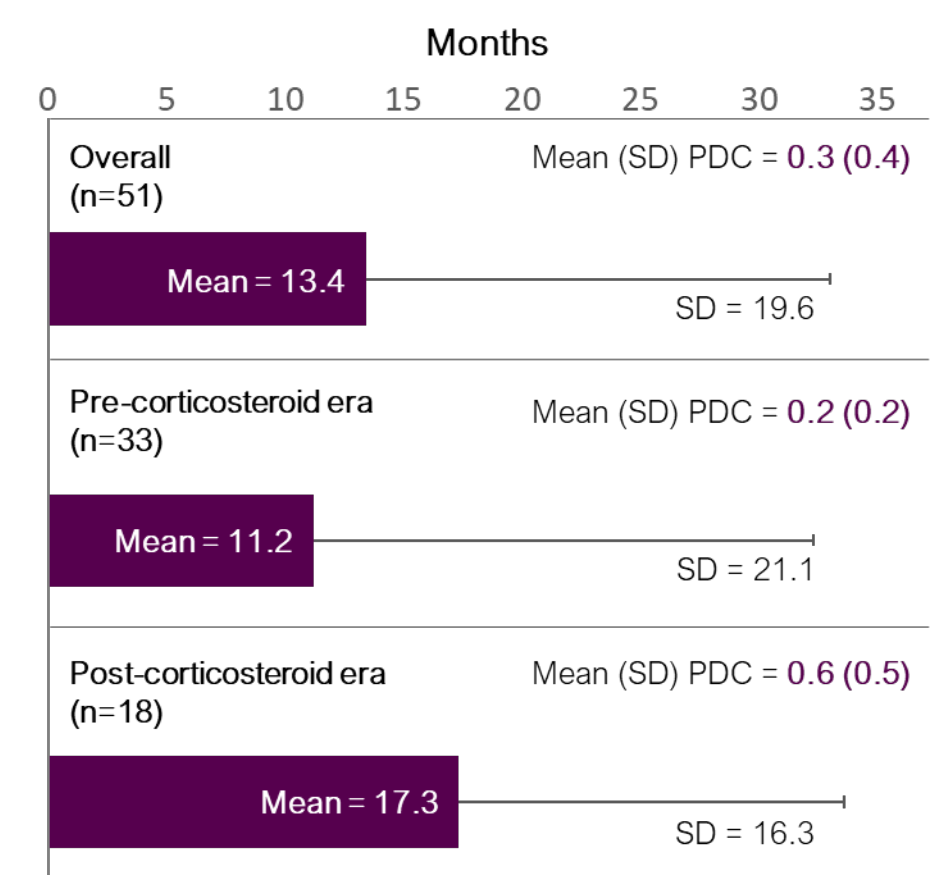


Figure 2. Mean PDC and duration of treatment, overall and among those in the pre- vs. post-corticosteroid era



DISCUSSION AND CONCLUSIONS

- These findings help describe corticosteroid treatment among real-world populations with DMD
 - Despite the long duration of follow-up, observed corticosteroid coverage per patient was relatively short. This may in part reflect limitations of the use of real-world administrative data for accurately describing corticosteroid treatment patterns.¹¹⁻¹²
 - Observed corticosteroid use was higher and was initiated earlier among those born during or after 2005, compared to those born before 2005.
 - The median age at initiation of corticosteroids among those born after 2005 was comparable to findings from the published literature.^{7,8}
- Strengths of the study include the real-world population-based nature of the dataset, the relatively closed nature of the population with little attrition,¹⁰ and the longevity of the data spanning the implementation of updated guidelines.
- Limitations include the lack of specific diagnostic codes for DMD (mitigated by using MD-specific codes with a clinical algorithm to identify the cohort), the relatively small sample size, that there was a special access program for deflazacort in place during the study period that could result in doses being missed in the data,¹³ and that data on those receiving healthcare outside of the provincial system might not be included.
- These data help to inform knowledge of the contemporary real-world healthcare and treatment patterns among DMD patients before and after the widespread use of corticosteroids. This study also informs the considerations and potential limitations for estimating treatment patterns in DMD patients using administrative data.

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