

SUPPLEMENTAL MATERIAL

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Supplemental Methods

Unlike the Brooke Scale, higher entry item values in the Performance of Upper Limb Module for DMD 1.2 represent greater functional ability rather than greater functional impairment. These are defined as ‘0 = no useful function of hands,’ ‘1 = can use hands to hold pen, pick up a coin or drive a powered chair,’ ‘2 = can raise 1 or 2 hands to mouth but cannot raise a cup with 200 g weight in it to mouth,’ ‘3 = can raise plastic cup with 200 g weight in it to mouth using 1 or 2 hands,’ ‘4 = can simultaneously raise both arms to shoulder height with elbow bent or in extension,’ ‘5 = can raise both arms simultaneously above head only by flexing the elbow,’ and ‘6 = can abduct both arms simultaneously with elbows in extension (without compensation) in a full circle until they touch above the head.’

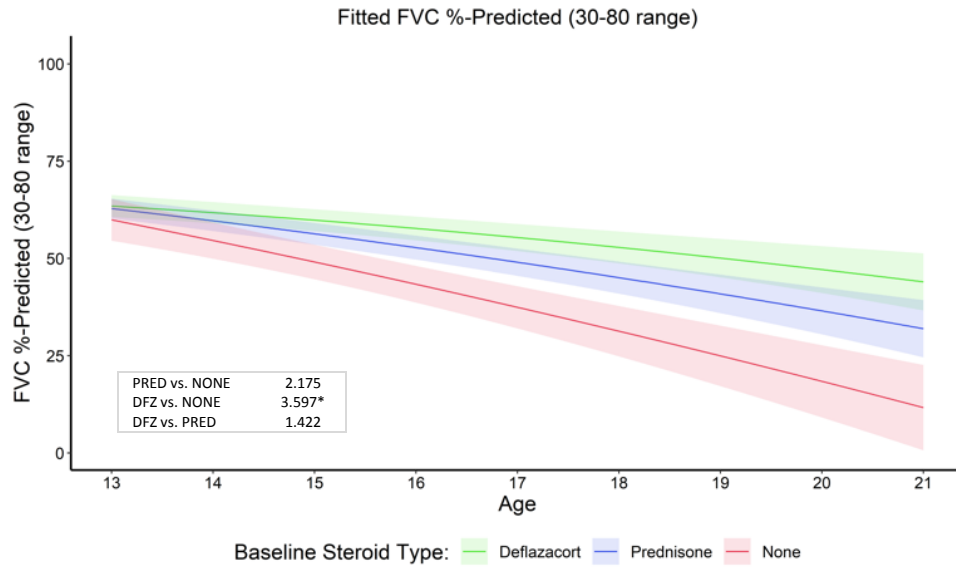
Supplemental Table 1. Baseline characteristics of non-ambulatory patients with DMD, by steroid use - additional measures

Mean \pm SD	Total N = 86	Prednisone n=29	Deflazacort n=40	None n=17	<i>p</i> -value ^a
Height Z-score	-1.28 \pm 2.05	-0.53 \pm 1.54	-2.13 \pm 2.32	-0.84 \pm 1.61	<0.01
Ability to transfer from a wheelchair	1.32 \pm 0.85	1.09 \pm 0.67	1.59 \pm 0.92	1.07 \pm 0.80	<0.05
FVC %-predicted (30-80 range) ^b	62.71 \pm 12.91	64.08 \pm 11.19	64.84 \pm 13.53	53.00 \pm 12.51	<0.05

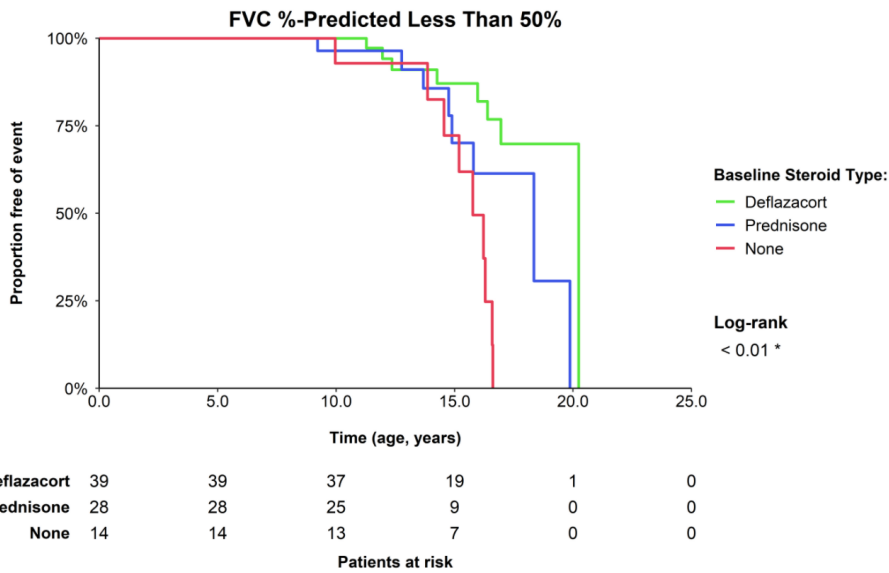
Abbreviations: BMI, body mass index; DMD, Duchenne muscular dystrophy; FVC, forced vital capacity; SD, standard deviation. Notes: ^a Reported *p*-values correspond to a comparison across all three steroid categories. ^b Sample sizes differ from overall sample as follows: n=25 (prednisone), n=25 (deflazacort), and n=9 (none).

Supplemental Figure 1. Longitudinal FVC %-predicted <50% (a) 30-80% (b) and <30% (c) among non-ambulatory patients with DMD, by steroid use

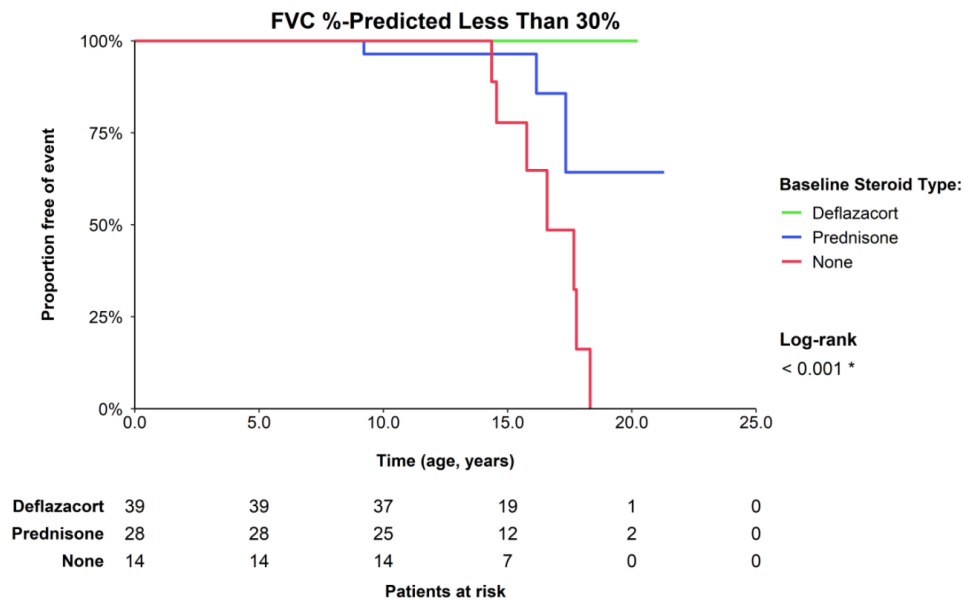
a.



b.

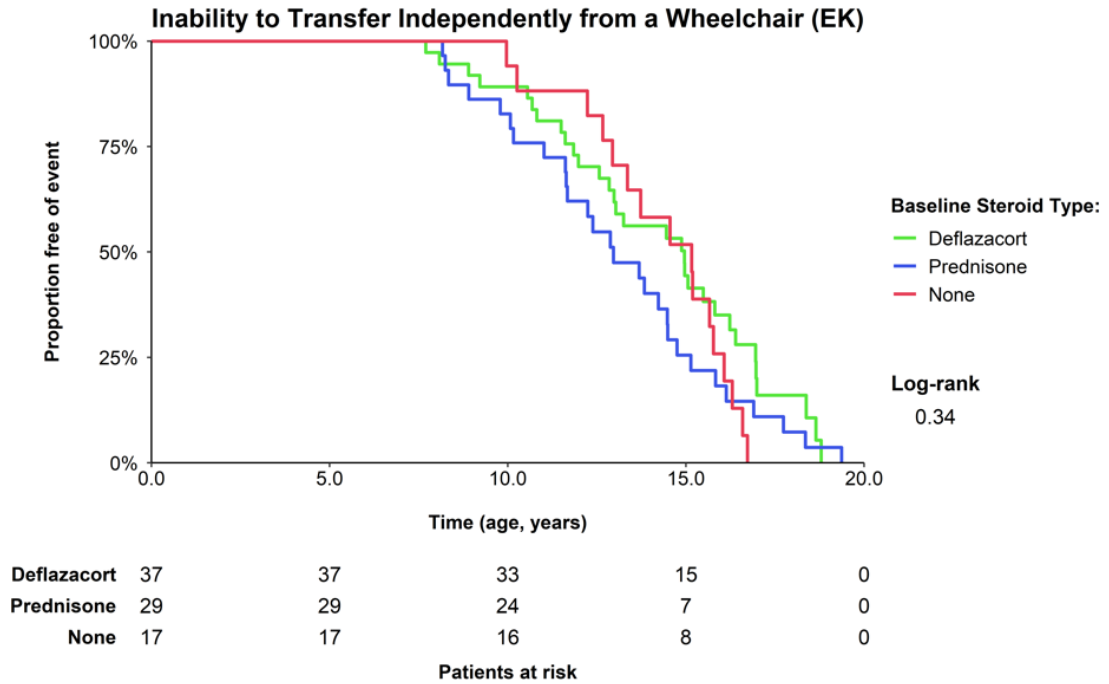


c.



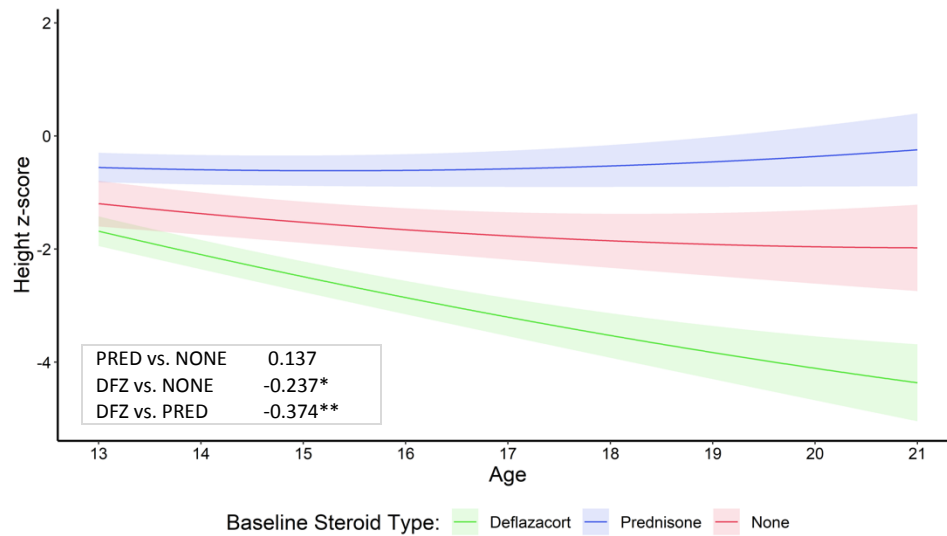
Caption: Shaded regions are bounded by ± 1 standard error. Estimates overlaid on plot are slope differences between steroid groups. Slope differences are obtained from two model specifications, which differ only by the baseline steroid reference group (none or prednisone). Abbreviations: DMD, Duchenne muscular dystrophy; FVC, forced vital capacity.

Supplemental Figure 2. Age at inability to transfer independently from a wheelchair among non-ambulatory patients with DMD, by steroid use



Abbreviations: DMD, Duchenne muscular dystrophy; EK, Egen Klassifikation.

Supplemental Figure 3. Height z-score among non-ambulatory patients with DMD, by steroid use



Caption: Shaded regions are bounded by ± 1 standard error. Estimates overlaid on plot are slope differences between steroid groups. Slope differences are obtained from two model specifications, which differ only by the baseline steroid reference group (none or prednisone). ** $p < 0.01$, * $p < 0.05$.

Abbreviations: DFZ, deflazacort; DMD, Duchenne muscular dystrophy; PRED, prednisone.