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INTRODUCTION & OBJECTIVE

- Duchenne muscular dystrophy (DMD) is a rare genetic disorder and primarily affects boys starting at a young age.¹ The combination of chronic muscle cell damage, inflammation, and fibrosis leads to several downstream complications, including muscle atrophy and weakness, cognitive impairment, and ultimately death due to respiratory failure or heart failure²
- DMD is frequently treated with corticosteroids/glucocorticoids (GCs) which are associated with side effects including stunted growth, bone comorbidities, weight increases, and infections.²⁻⁴ The two most prescribed GCs in DMD patients are prednisone and deflazacort.⁴
- This study aims to understand the holistic disease burden and treatment experiences for DMD patients despite the use of GCs.

METHODS

- A web-enabled, retrospective medical chart audit, approved by an independent Institutional Review Board, was conducted with qualifying physicians between October-November 2022.
- Forty-six eligible physicians (neurologists, cardiologists, and endocrinologists) who each manage at least 10 DMD patients participated and provided a total of 103 charts for DMD patients (Table 1).
- Patient charts were eligible if the patient was currently receiving Standard of Care (SOC) GCs for at least 12 months or the patient discontinued GC use within the past 12 months.
- The study instrument contained a case report form to capture patient chart-level data (patient demographics, comorbidities, treatment experience, symptoms, healthcare resource utilization) and a physician-level survey.

TABLE 1: PATIENT CHART AND PHYSICIAN CHARACTERISTICS

Primary Physician Medical Specialty

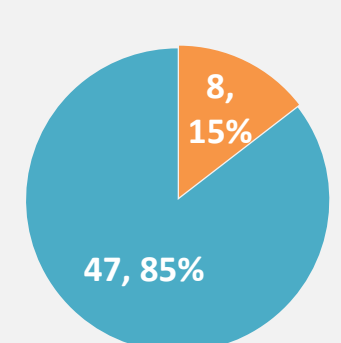
Neurologists	Cardiologists	Endocrinologists	Total
37	5	4	46

Patient Chart-Level Data

Patient Charts	Total
Total	103
Age Distribution	
2-7 years, n (%)	47 (46%)
8-12 years, n (%)	30 (29%)
13-18 years, n (%)	20 (19%)
>18 years, n (%)	6 (6%)
Glucocorticoid Experience	
Currently receiving glucocorticoids, n (%)	100 (97%)
• Prednisone/prednisolone, n (%)	55 (53%)
• Deflazacort, n (%)	45 (44%)
Discontinued glucocorticoid in the past 12 months, n (%)	3 (3%)

PATIENT CHART-LEVEL RESULTS

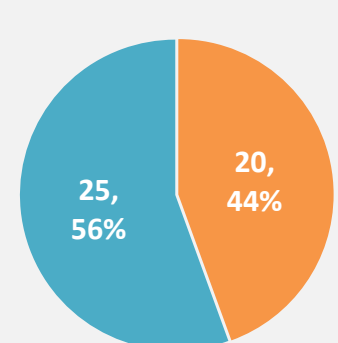
Treatment History of Current Prednisone Users (n, %) N= 55 charts



8, 15%
47, 85%

Previously treated with deflazacort
Was glucocorticoid naive

Treatment History of Current Deflazacort Users (n, %) N= 45 charts

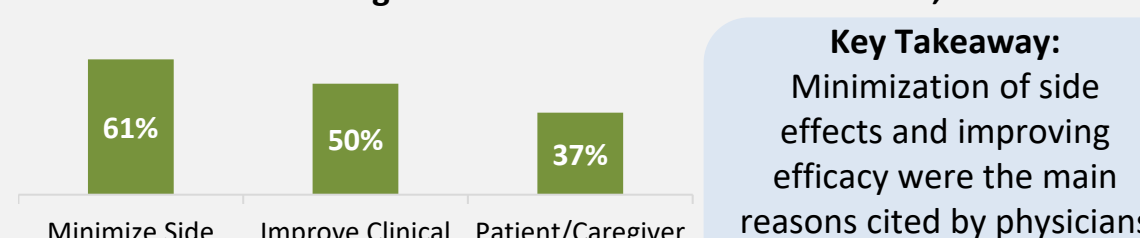


25, 56%
20, 44%

Previously treated with prednisone
Was glucocorticoid naive

Key Takeaway: While most patients currently taking prednisone were not previously treated with deflazacort (85%), over half of those currently taking deflazacort were not previously treated with prednisone (56%).

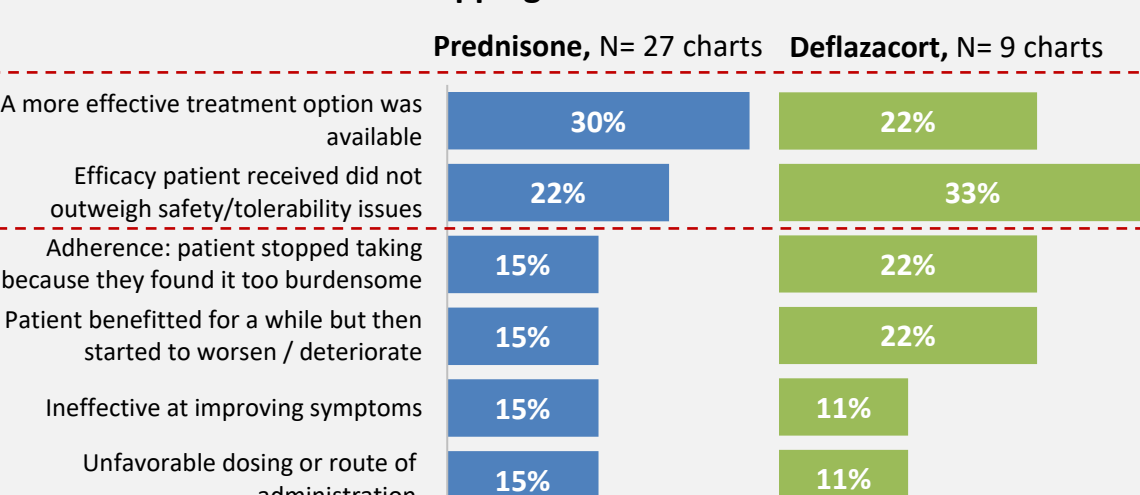
Reasons for Prescribing Deflazacort Instead of Prednisone*, N= 54 charts



*Response percentages exceed 100% because the question allowed physicians to select multiple responses

Key Takeaway: Minimization of side effects and improving efficacy were the main reasons cited by physicians for avoiding prednisone

Rationale for Stopping Prednisone and Deflazacort*

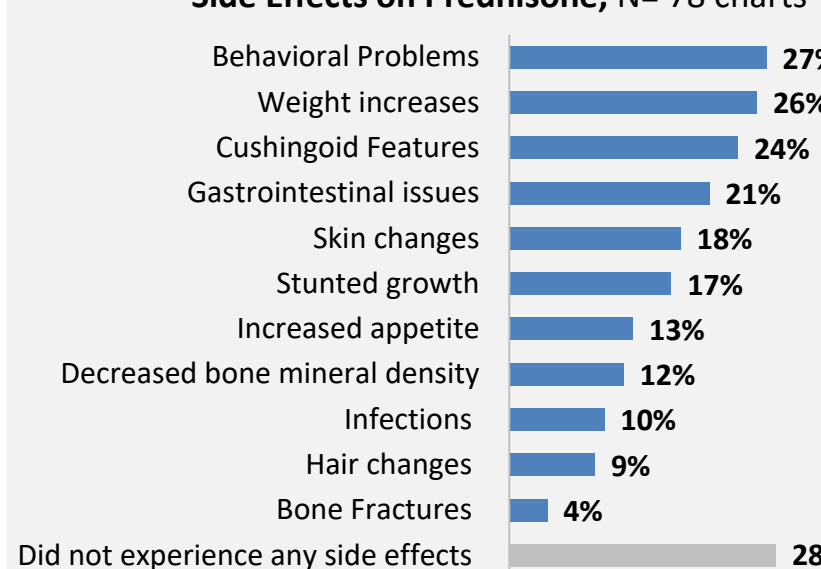


*Response percentages exceed 100% because the question allowed physicians to select multiple responses

Key Takeaway: The top reason to stop prednisone was the availability of a more effective treatment, while the top reason to stop deflazacort was efficacy did not outweigh safety/tolerability issues.

PREDNISONE

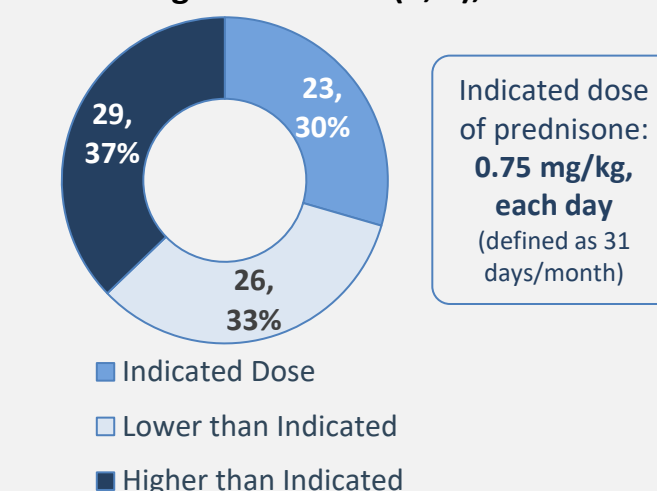
Side Effects on Prednisone, N= 78 charts*



*N= 55 charts were current prednisone users; N= 23 charts were prior prednisone users

Key Takeaway: Most patients (72% of prednisone users; 54% of deflazacort users) experienced side effects (SEs). Stunted growth and behavioral problems were common SE (≥15%) experienced by patients on both prednisone and deflazacort. Growth/bone health-related SEs were also common across patients on prednisone and deflazacort.

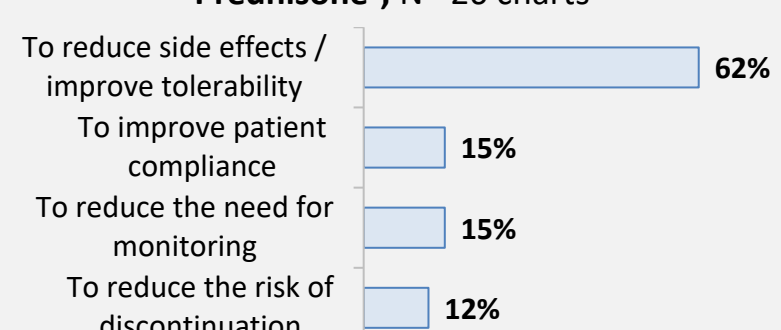
Prednisone Dosing Distribution (n, %), N= 78 charts



Indicated dose of prednisone: 0.75 mg/kg, each day (defined as 31 days/month)

■ Indicated Dose
■ Lower than Indicated
■ Higher than Indicated

Rationale for Lower/Less Frequent Dose of Prednisone*, N= 26 charts

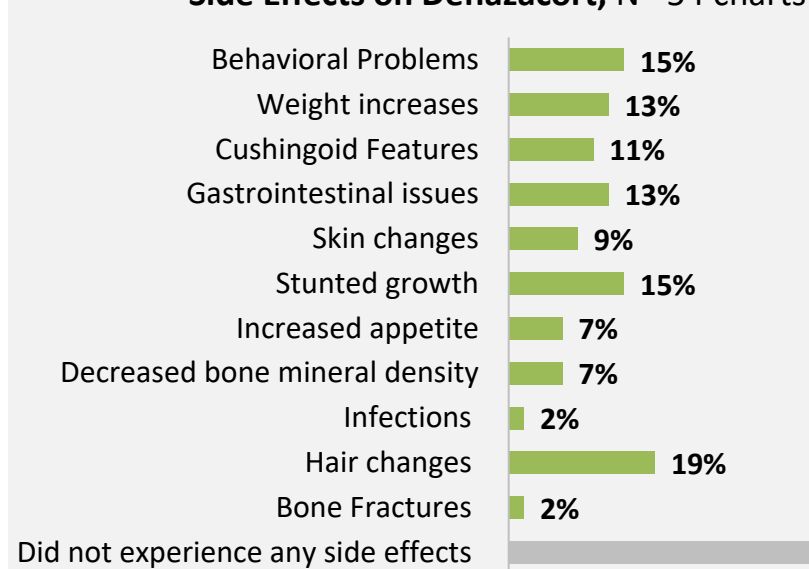


*Response percentages exceed 100% because the question allowed physicians to select multiple responses

Key Takeaway: 33% of prednisone users and 46% of deflazacort users were prescribed lower / less frequent doses than indicated. The top reason physicians reported was to reduce side effects/improve tolerability (62% and 64%, respectively).

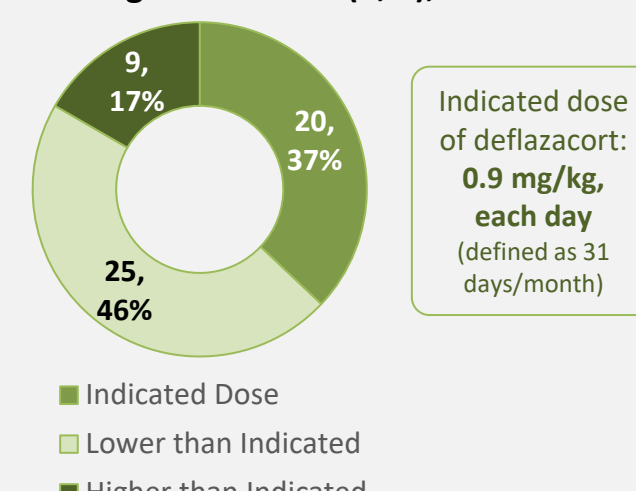
DEFLAZACORT

Side Effects on Deflazacort, N= 54 charts*



*N= 45 charts were current deflazacort users; N= 9 charts were prior deflazacort users

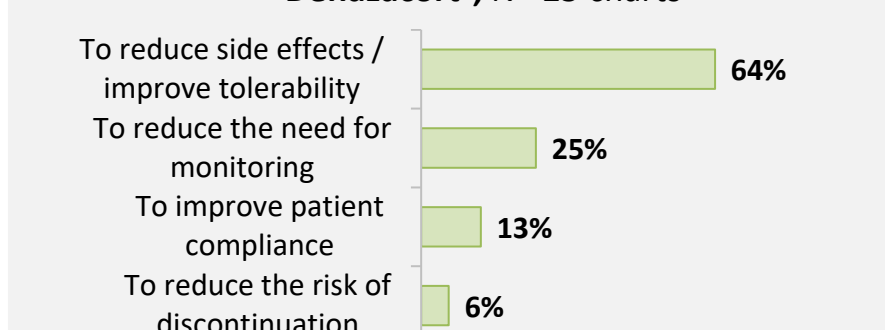
Deflazacort Dosing Distribution (n, %), N= 54 charts



Indicated dose of deflazacort: 0.9 mg/kg, each day (defined as 31 days/month)

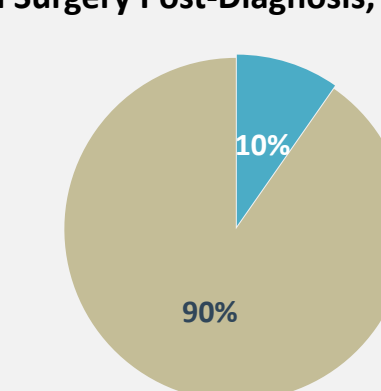
■ Indicated Dose
■ Lower than Indicated
■ Higher than Indicated

Rationale for Lower/Less Frequent Dose of Deflazacort*, N= 25 charts



*Response percentages exceed 100% because the question allowed physicians to select multiple responses

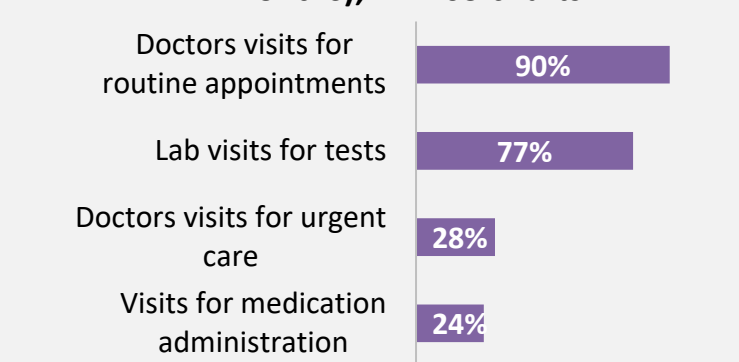
Proportion of Patient Charts with DMD-related Surgery Post-Diagnosis, N= 103 charts



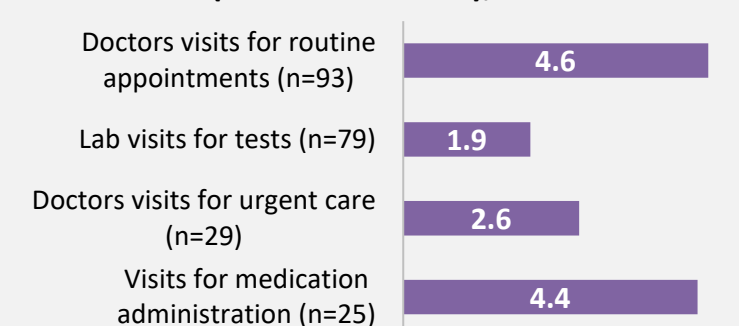
10%
90%

Had surgery post-diagnosis
Did not have surgery post-diagnosis

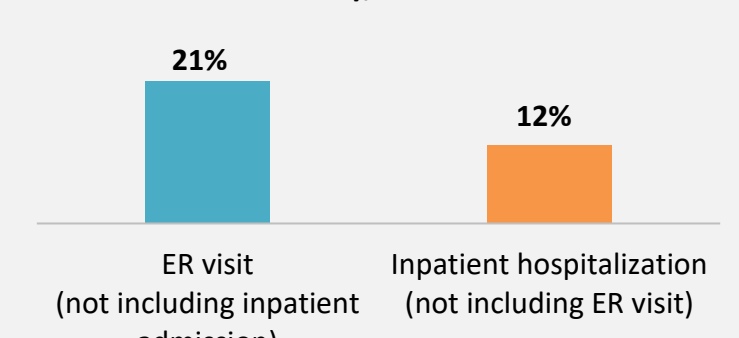
Proportion of Patient Charts with DMD-related Outpatient or Lab Visit (Prior 12 Months), N= 103 charts



Average Number of DMD-related Outpatient or Lab Visit (Prior 12 Months), N= 103 charts



Proportion of Patient Charts with DMD-related Emergency Room (ER) and Inpatient Visit (Prior 12 Months), N= 103 charts



DISCUSSION & CONCLUSIONS

- This medical chart audit study provides real-world evidence on the treatment patterns and experiences, and healthcare resource utilization (HCRU) of patients with DMD treated with GCs.
- Additionally, this study provides insight into the clinician rationale for treatment decisions and perceived unmet needs for patients with DMD.
- Patients taking both prednisone and deflazacort are prescribed sub-therapeutic doses, often in an attempt to reduce side effects / improve tolerability. This suggests that some patients may not experience the intended therapeutic benefit, further driving clinical burden and HCRU.
- This study highlights the significant unmet need for an effective therapy that can deliver comparable efficacy as SOC GCs, but with an improved safety / tolerability profile.**

ACKNOWLEDGMENTS

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