Patient and Caregiver Treatment Preferences in Duchenne Muscular Dystrophy: A Targeted Literature Review

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KEY MESSAGE

"Across all three included studies assessing treatment preferences in DMD, patients and caregivers were willing to accept treatment risks or uncertainty in exchange for improved muscle function or slower disease progression."

1 BACKGROUND

Duchenne muscular dystrophy (DMD) is a progressive X-linked neuromuscular disorder characterised by muscle weakness and multisystem complications, with symptoms typically emerging in early childhood between ages 2 and 5.[1,2]

Estimated global birth prevalence is 19.8 per 100,000 live male births; while the global prevalence is 7.1 per 100,000 males.[3]

Progressive muscle weakness can compromise mobility, cardiac and respiratory function, and independence.[1,4-6]

- Current disease-modifying and supportive care can slow decline, but DMD remains progressive and life-limiting, with lasting effects on patients and caregivers.[1-2,4-6]
- As new treatments emerge, understanding how patients and caregivers weigh treatment benefits, risks, and uncertainty is important to inform treatment development and decisions.[7-10]

2 OBJECTIVES

This review set out to identify and summarise studies using discrete choice experiments (DCEs) or conjoint analysis to assess preferences for DMD treatments among patients and/or caregivers. Four questions guided the synthesis.

1. Identify studies using DCE or conjoint analysis to assess DMD treatment preferences
2. Describe the treatment attributes assessed
3. Summarise the preference patterns reported
4. Report differences between respondent groups

3 METHODS

A targeted literature review was conducted in Embase, supplemented by a desk search, to identify relevant evidence published up to June 2026. The flow on the left shows how records moved from search to synthesis (**Figure 1**); the criteria on the right define what was in scope (**Figure 2**).

Figure 1: PRISMA flow diagram

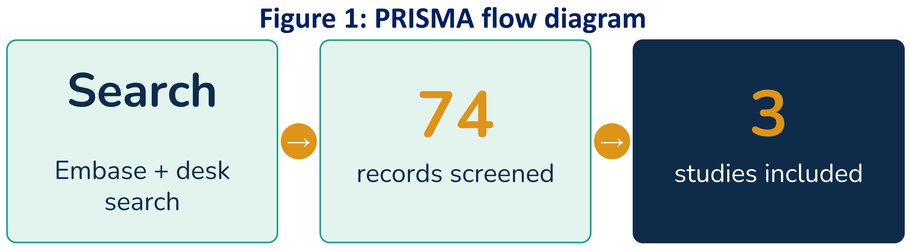
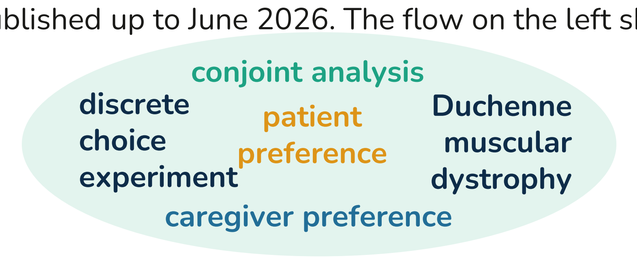


Figure 2: Search terms



Eligibility: patient and/or caregiver preferences for DMD treatments elicited with DCE or conjoint analysis.

4 RESULTS

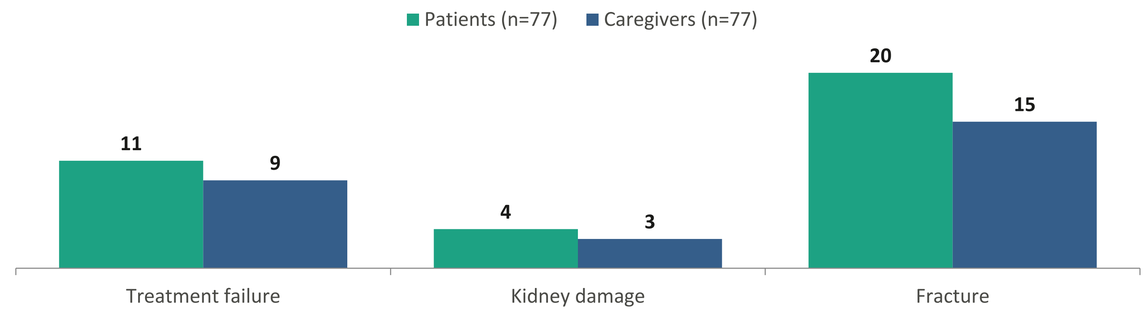
Three independent studies met the predefined methodological criteria: two involving US-based populations and one conducted across eight countries. Two used DCEs; the third assessed conjoint analysis and best-worst scaling (BWS) in the same survey. Attributes were developed using literature review, advisory-board input and/or collaboration with patients, caregivers, clinicians and other stakeholders **(Table 1, Figures 3–4).**

Table 1: Key findings from the included studies

Study	Method and setting	Respondents	Attributes	Main findings
Hollin et al. 2015[7]	Conjoint analysis and BWS; US	119 caregivers of children with DMD. Mean age: caregivers: 43.7 years; patients: 12.1 years	Muscle function effect, lifespan gain, post-approval drug information, nausea, bleeding risk, heart-arrhythmia risk	Among caregivers, the effect of treatment on muscle function had the highest conditional attribute importance (26.9%), followed by arrhythmia risk (23.9%). BWS and conjoint analysis showed significant agreement in treatment rankings ($p < 0.01$). (**Figure 4**)
Bridges et al. 2018[8]	DCE (12 choice tasks); US	161 respondents (Duchene and Becker muscular dystrophy): 9 patients, 87 caregivers, 65 professionals (mean ages: 23.7, 44.8, and 45.7 years, respectively)	Muscle-strength improvement, treatment failure uncertainty, kidney-damage risk, fracture risk	Respondents preferred improved muscle strength (Coef = 0.88). For improved muscle function, respondents accepted up to 22% increased uncertainty in benefit, 9% risk of kidney damage, or 26% increased fracture risk (all $p < 0.001$). Patients were less risk-tolerant than caregivers and professionals ($p<0.001$)
Crossnohere et al. 2022[9]	DCE; 8 countries	154: 77 adults with DMD, 77 caregivers of adults. Mean patient age: 25.3 years. Caregivers and patients were matched based on patient age.	Duration of disease slowing, treatment failure uncertainty, kidney-damage risk, fracture risk	No significant patient–caregiver differences in MAR; 99% preferred the profile with 3 years of slowed progression, a 25% treatment-failure rate, 10% kidney-damage risk, and no fracture risk over the profile with one year of slowing, a 75% failure rate, and 20% risks of kidney damage and fracture. (**Figure 3**)

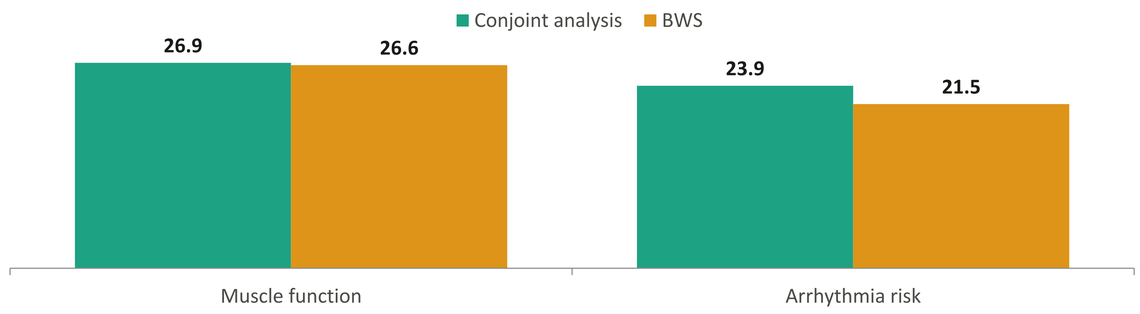
All three studies found respondents willing to accept risk or uncertainty in exchange for preserved muscle function or slower progression.

Figure 3: Crossnohere et al. 2022: MAR (%) for one year of disease slowing



Patients and caregiver MAR estimates did not differ significantly (p=0.267, p=0.719, p=0.534).

Figure 4: Hollin et al. 2015: conditional attribute importance (%)



Both methods ranked muscle function first and arrhythmia risk second.

5 DISCUSSION

Across these studies, muscle function or slower disease progression was an important treatment benefit, and respondents weighed it against treatment failure and safety risks.

Findings on patient–caregiver preferences differed between the two DCEs. The small US patient subgroup (n=9) was less risk-tolerant than caregivers and healthcare professionals, whereas the larger age-matched international study found no statistically significant differences between patients and caregivers.

These differences should be interpreted in the context of varying sample sizes, study populations, treatment attributes and benefit–risk scenarios. The limited number of studies and differences in treatment attributes and methods highlight the need for further preference research to better understand patient and caregiver priorities in DMD.

6 CONCLUSION

Evidence suggests that treatment efficacy, particularly improved muscle function or slower disease progression, was a central preference driver. Respondents also considered treatment failure, safety risks, lifespan, and other treatment characteristics.

- The small number of eligible studies, limited patient representation in some samples and differences in study scenarios limit cross-study comparisons.
- Future research should aim to recruit more patients where feasible to better capture their perspectives, while recognising the recruitment challenges associated with DMD.

Abbreviations: BWS, best-worst scaling; DBMD, Duchenne and Becker muscular dystrophy; DCE, discrete choice experiment; DMD, Duchenne muscular dystrophy; MAR, maximum acceptable risk.

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REFERENCES

1. Duan D, et al. Duchenne muscular dystrophy. Nat Rev Dis Primers. 2021;7(1):13.
2. Darras BT, Urion DK, Ghosh PS. Dystrophinopathies. GeneReviews. Updated 2022.
3. Crisafulli S, et al. Global epidemiology of DMD: a systematic review and meta-analysis. Orphanet J Rare Dis. 2020;15(1):141.
4. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1. Lancet Neurol. 2018;17(3):251–267.
5. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2. Lancet Neurol. 2018;17(4):347–361.
6. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3. Lancet Neurol. 2018;17(5):445–455.
7. Hollin IL, Peay HL, Bridges JFP. Caregiver preferences for emerging DMD treatments: a comparison of BWS and conjoint analysis. Patient. 2015;8(1):19–27.
8. Bridges JFP, Tsai JH, Janssen E, et al. How do members of the Duchenne and Becker muscular dystrophy community perceive a DCE incorporating uncertain treatment benefit? Patient. 2019;12(2):247–257.
9. Crossnohere NL, Fischer R, Vroom E, Furlong P, Bridges JFP. A comparison of caregiver and patient preferences for treating DMD. Patient. 2022;15(5):577–588.
10. Landfeldt E, Edström J, Lindgren P, Lochmüller H. Patient preferences for treatments of neuromuscular diseases: a systematic literature review. J Neuromuscul Dis. 2017;4(4):319–329.

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