

Glucocorticoid side-effects in Duchenne Muscular Dystrophy: Systematic review of side-effects in published literature and a survey of the concerns of the patient community

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INTRODUCTION AND AIMS

Duchenne Muscular Dystrophy (DMD) is a rare, X-linked, muscle-wasting disorder with Glucocorticoids (GC) used as disease-modifying therapy. Long-term GC therapy is associated with a range of side-effects.

This project aimed to evaluate side-effects of GC use in young people with DMD in published research (clinician priority) and those of concern to young people with DMD and their families (patient priority)

METHODS

A systematic review was conducted with three databases - MEDLINE, EMBASE and the Cochrane Library systematically searched for publications.

An anonymous online survey was developed and aimed at parents and carers of a boy with DMD.

The survey assessed:

- Current and previous GC use
- Perception of GC side-effects
- GC side-effects experienced

Inclusion Criteria	Exclusion Criteria
Males with DMD	Studies with no GC-only arm
GC treatment	Animal studies
Any report of known GC side-effects	Studies with female participants
Primary research papers	Primary research papers with less than 3 participants
	Conference abstracts and letters
	Studies combining patients with DMD with other muscular dystrophies

Table 1: Inclusion and Exclusion Criteria for Systematic Review

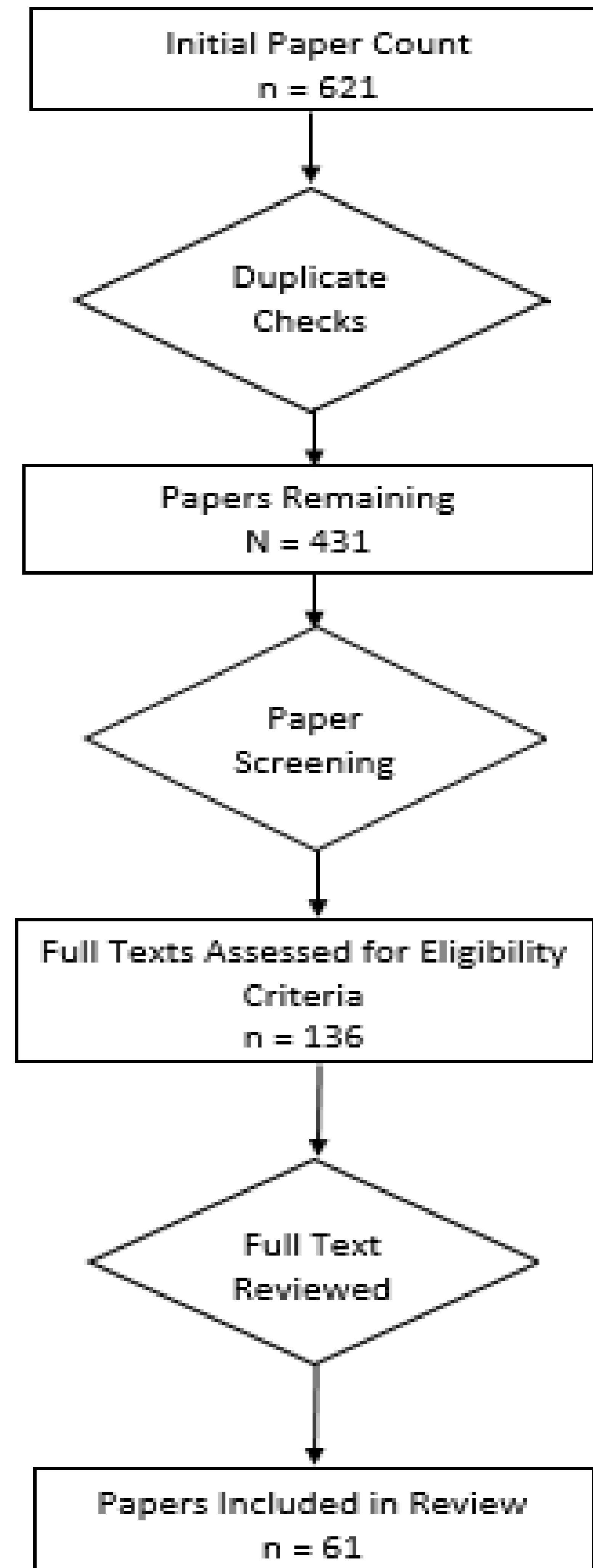


Figure 1: PRISMA Diagram: Simplified paper selection process

SURVEY RESPONSES (demographics)

The survey was live between February 2021 and March 2021.

Thirty-three responses were included.. Thirty-two (97%) of the respondents were parents/guardians and the median age of the young people was 10 (Q1 =6, Q3 = 13) Thirty-one (93.9%) of the young people were currently taking GC.

RESULTS (systematic review)

Top 5 Side Effects (Sought For)

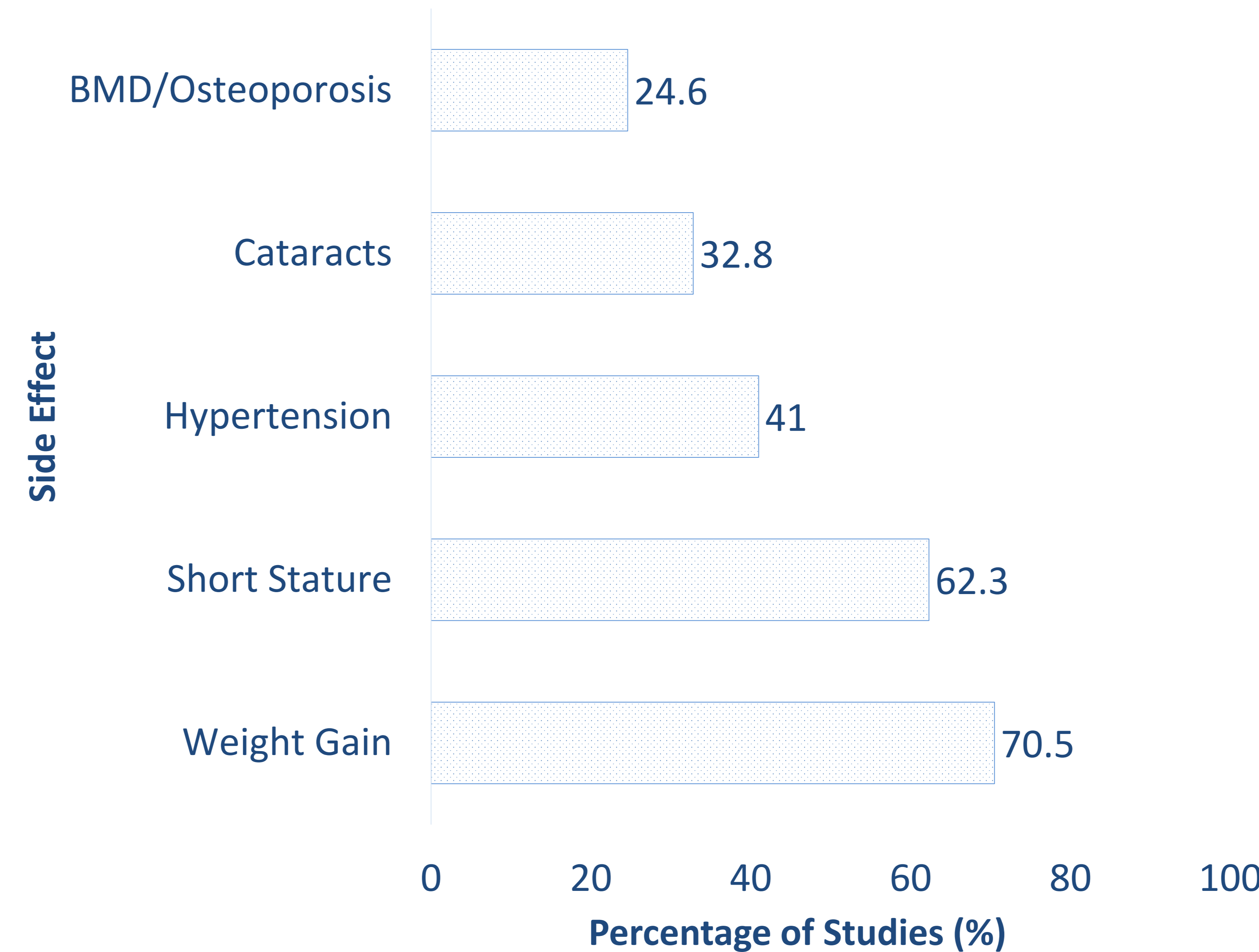


Figure 2: Top 5 Side-Effects Sought For in Published Literature. BMD = Bone Mineral Density

RESULTS (survey)

Top 5 Side Effects of Concern to Carers

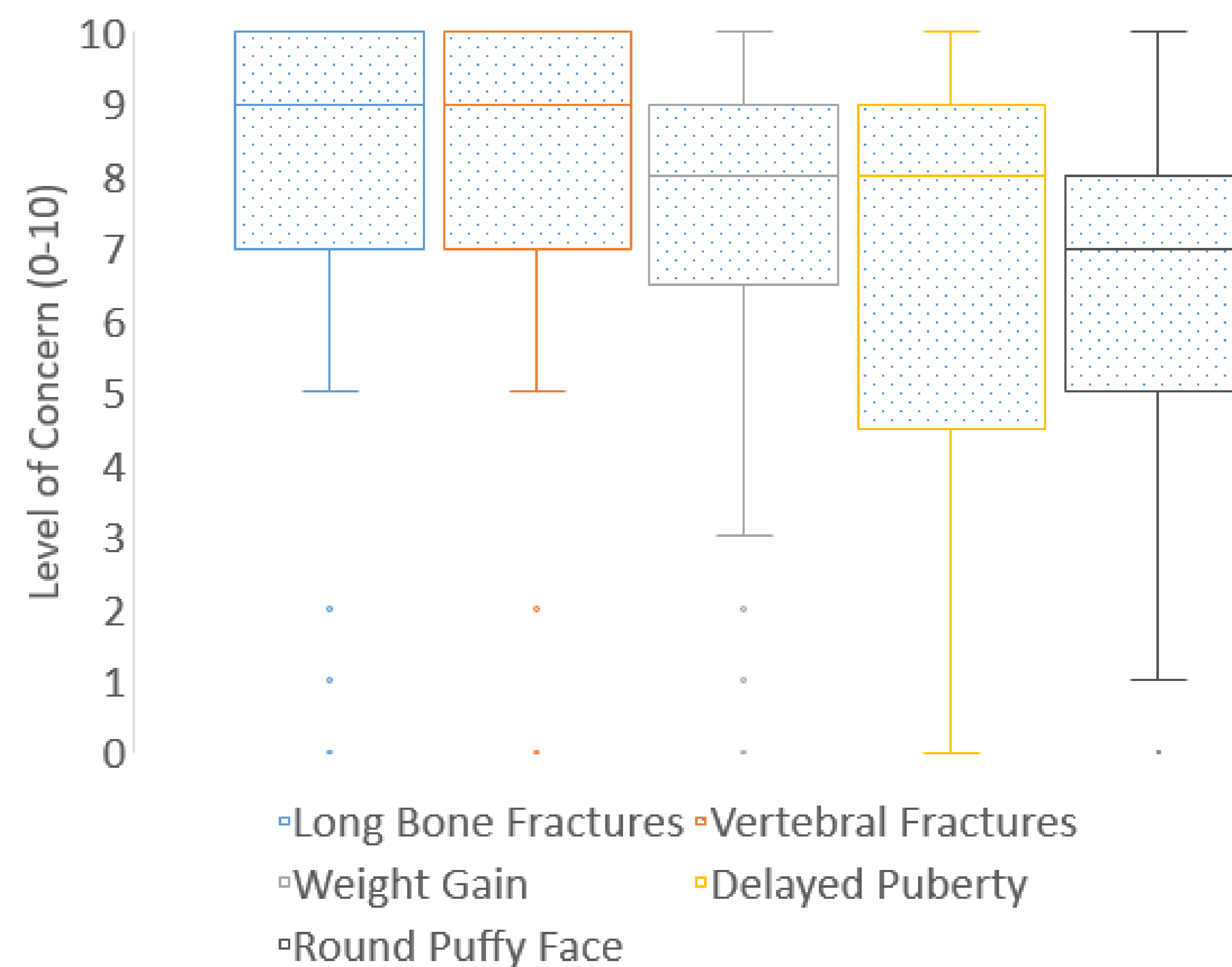


Figure 3: Top 5 Side-effects of Concern to Carers

Sixty-one publications were included in the systematic review, with fifty-three side-effects extracted. Weight gain was the side-effect of highest clinician priority (70.5%) followed by short stature (62.3%) and hypertension (41%).

RESULTS (survey)

Top 5 Side-effects of Concern to Young Person

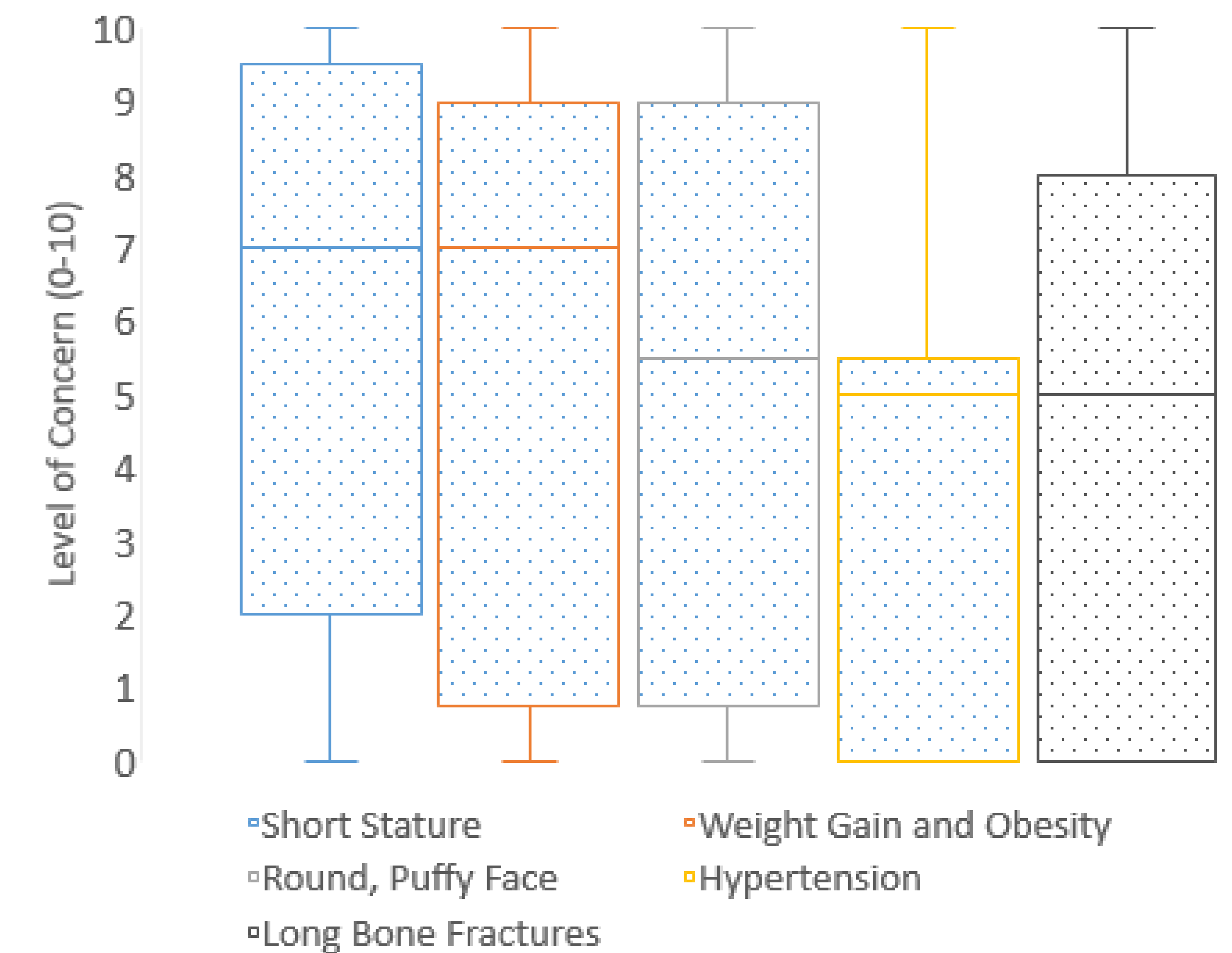


Figure 4: Top 5 Side-Effects of Concern to Young Person with DMD

CONCLUSIONS

- Most GC side effects of concern to carers and young people with DMD are addressed in published research but there is very limited information on disorders of puberty in this population.
- Our survey identified the GC side effects which concern carers and young people with DMD for the first time.
- Endocrine and bone GC side effects are of great concern to carers and young people with DMD and justifies research in this area.

ACKNOWLEDGEMENTS

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