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

Caleb Hariri and Sze Choong Wong, Department of Paediatric Endocrinology, Royal Hospital for Children, University of Glasgow, 1345 Govan Road, G51 4TF, Glasgow

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

RESULTS (systematic review)

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)

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
We would like to thank the following organisations for their help in circulating the survey amongst their members:
- Duchenne UK, Action Duchenne, Muscular Dystrophy UK, Duchenne Family Support Group, and Neuromuscular Pathfinders Alliance