

Variability of advice and education for steroid sick day dosing in Duchenne Muscular Dystrophy: A UK wide patient survey

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INTRODUCTION AND AIMS

The use of long term oral glucocorticoid (GC) in DMD is associated with a range of endocrine side effects including secondary adrenal insufficiency. The updated international care consensus for DMD (2018) recognizes this important issue and recommends emergency plans to be in place.



Making sure every DMD patient in the UK has access to the best care

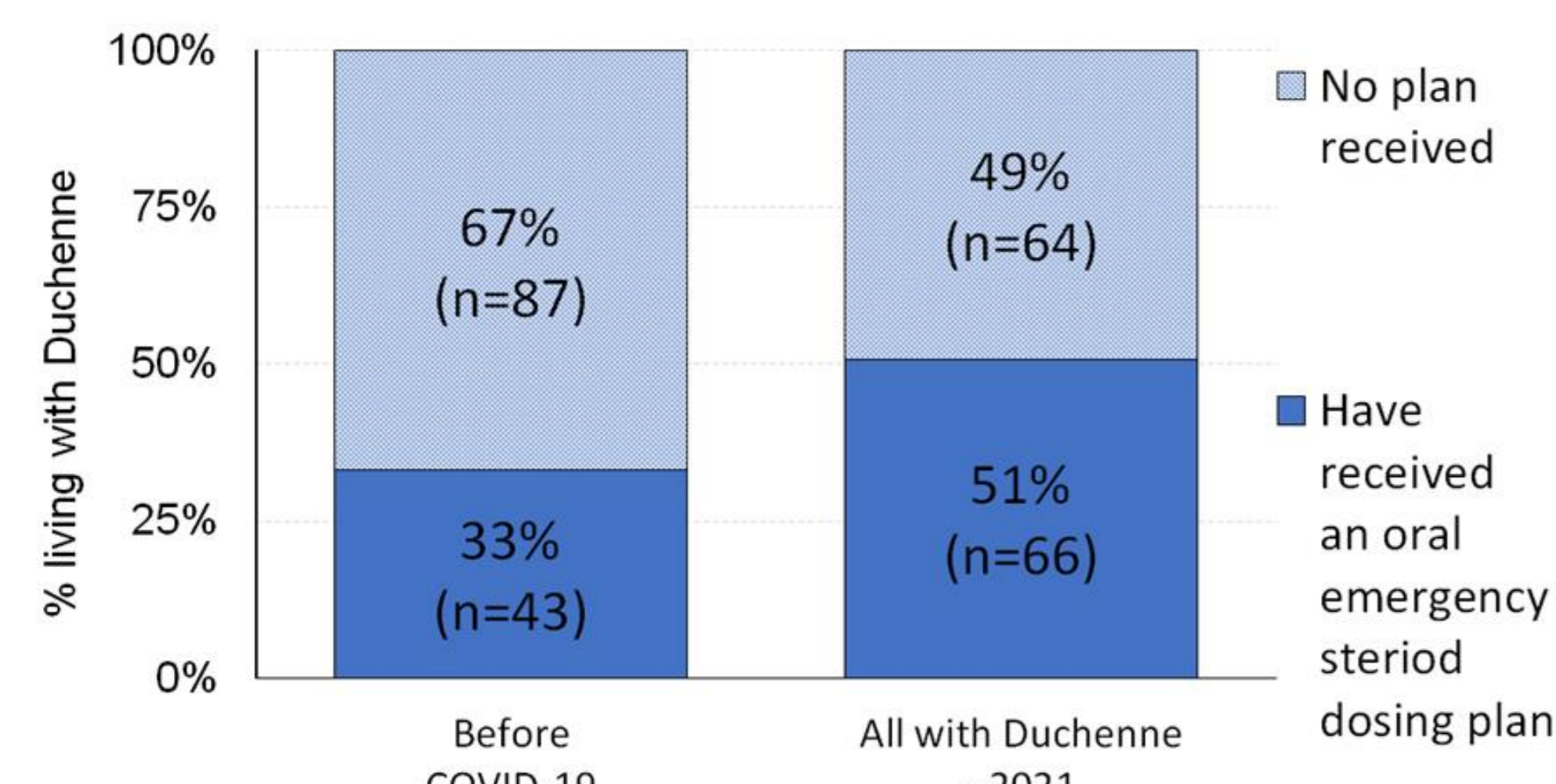
In the UK, DMD Care UK (www.dmdcareuk.org) a project was launched in 2020 to facilitate implementation of standards of care in all aspects of care of DMD. This survey is conducted as part of the project and aims to determine the advice and education of sick day dosing plans currently given to families of boys with DMD and to identify the needs of families.

METHODS

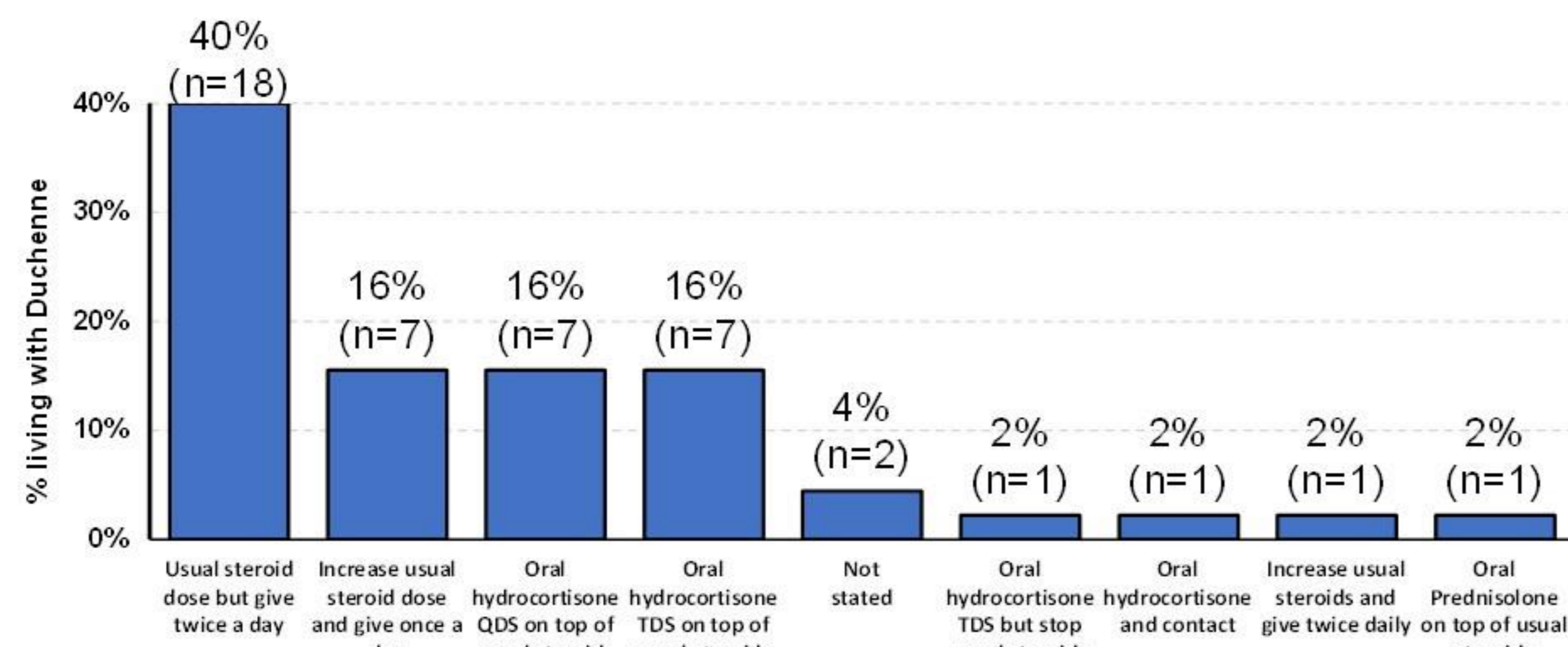
An online survey was developed by the research team (SCW, AJ, ST, TM, MG) and was reviewed by carers of a young person with DMD, adolescents/adults with DMD from a clinic in Glasgow and as part of the patient advisory board of Duchenne UK. The survey was distributed online by Duchenne UK between November 2020 and January 2021. Results are presented as median (range).

RESULTS

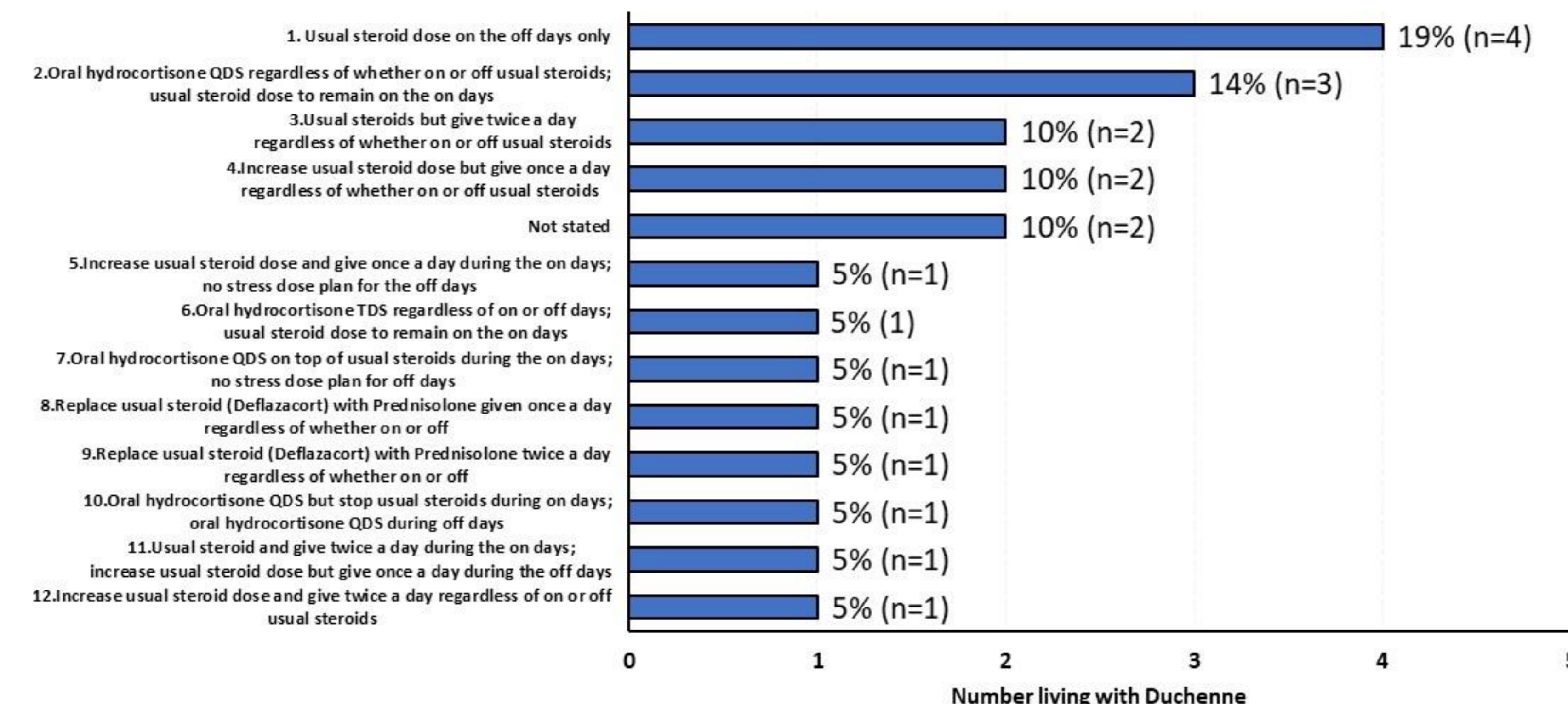
130 responses were obtained from 129 carers of a person with DMD (one parent provided responses for twins). Median age of the person with DMD was 11.5 years (5, 26). All were on GC therapy with 83/130 (64%) on daily therapy.



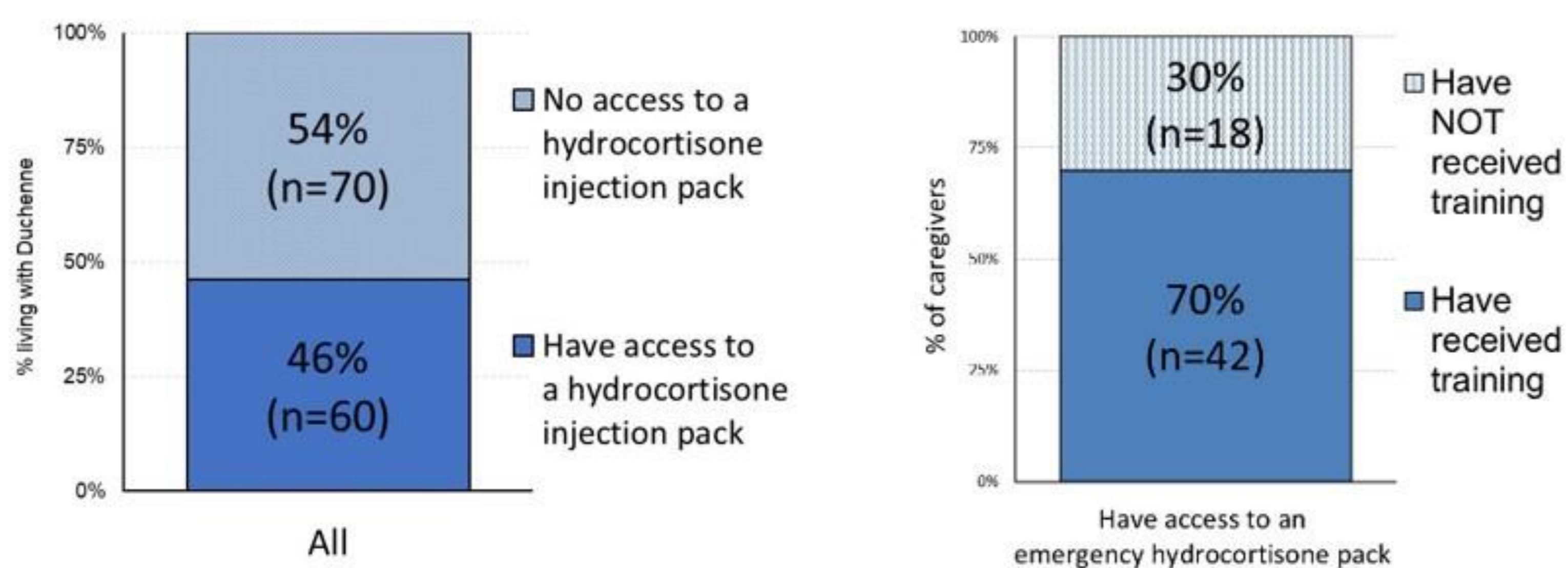
Almost half of boys (51%) with DMD had an oral stress dose plan in place for prevention of adrenal crisis with mild acute illness.



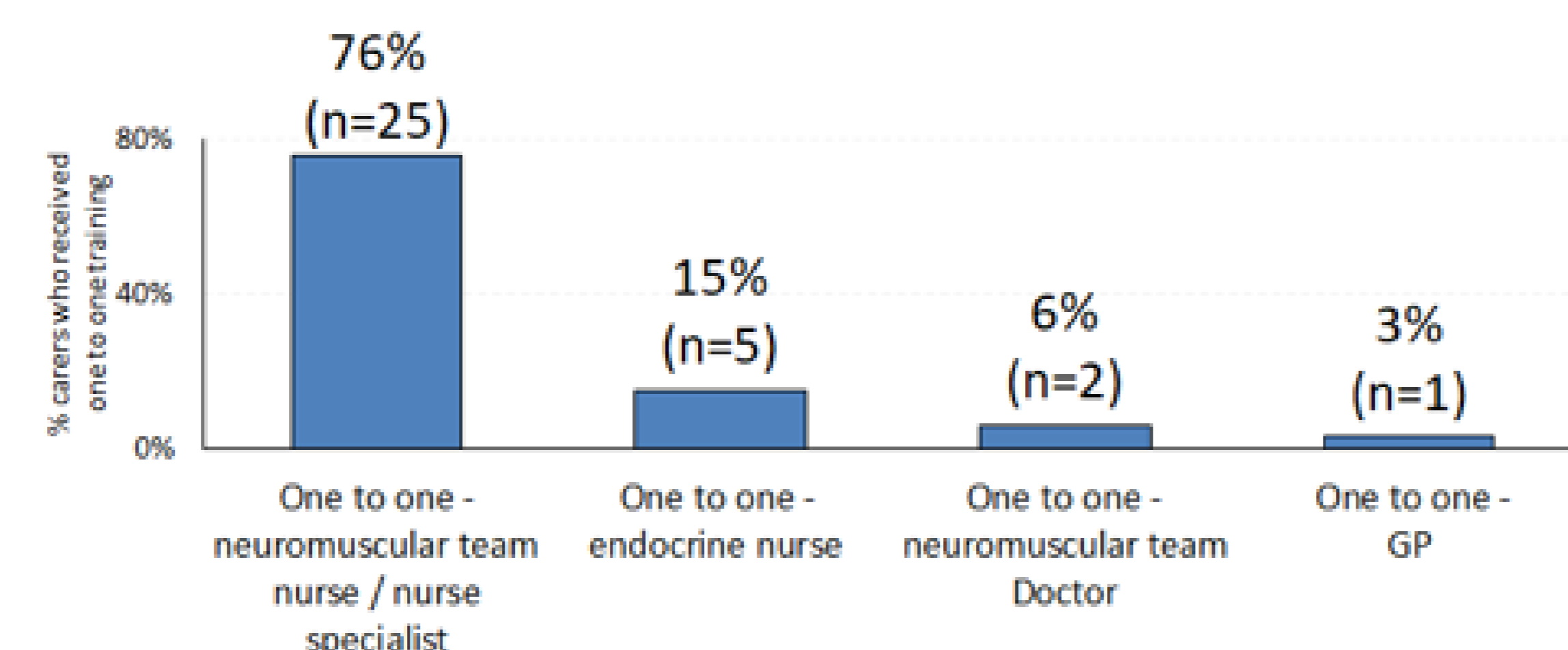
A total of 8 different oral stress dose plans were identified for those on daily therapy.



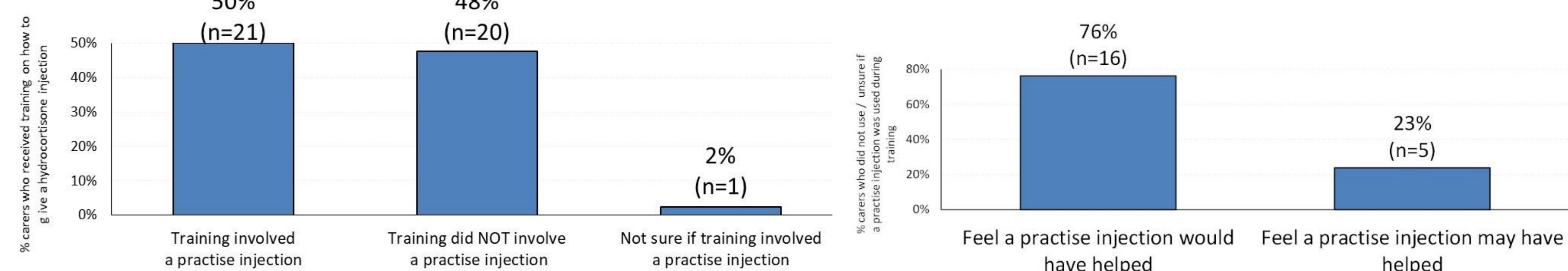
A total of 12 different oral stress dose plans were identified for those on intermittent therapy.



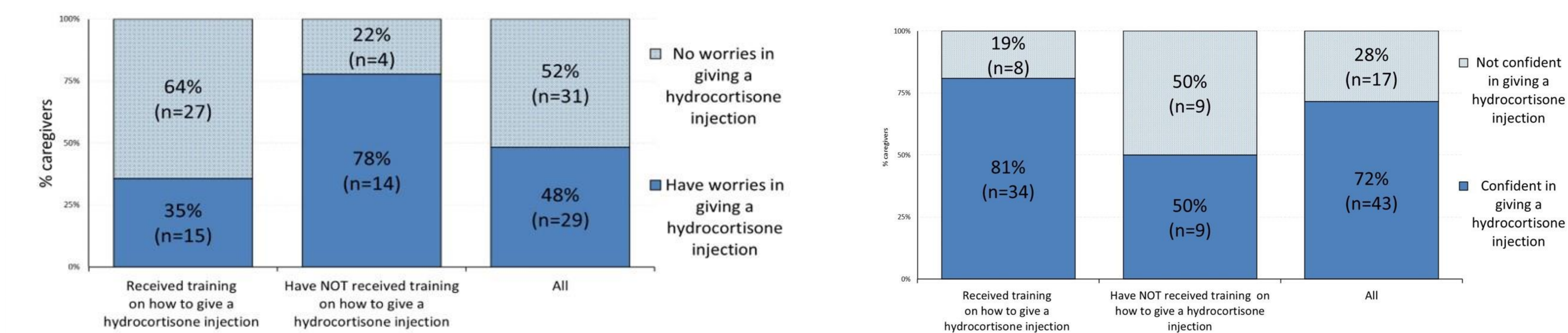
Under half (46%) had access to hydrocortisone for injection at home in case of severe illness/impending adrenal crisis. A large proportion (70%) of those with access to hydrocortisone had received information/training.



Training was mostly provided by the neuromuscular team.



Only half (50%) of those who were trained had a practise injection. Almost all (99%) who did had the opportunity to perform a practise injection that that it would help or it may help.



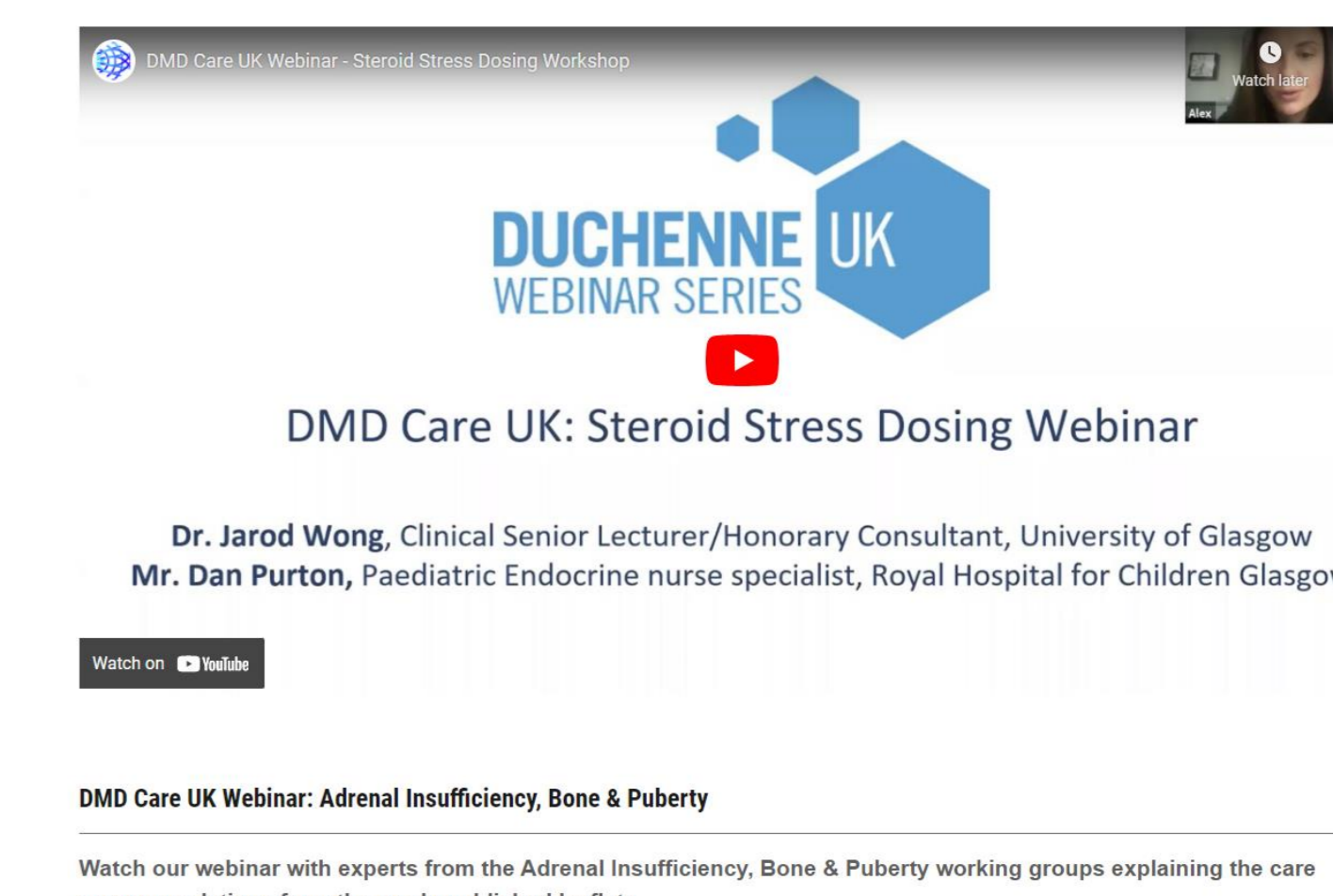
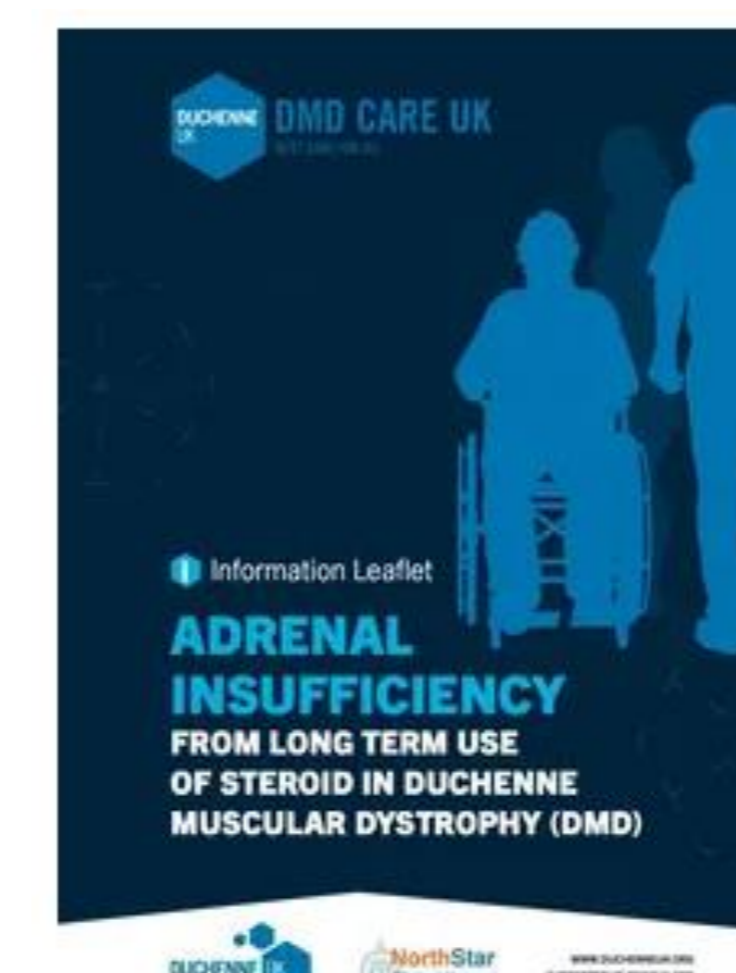
Carers who had a practise injection as part of training were less worried and more confident about giving an injection.

CONCLUSION

Despite the known risk of adrenal crisis in patients on long term high dose GC therapy and recommendations of the recent international care consensus in DMD (2018), emergency sick day plans were not consistently in place in this national survey of boys with DMD. Solutions are needed for implementation of this aspect of care, but could be challenging and must take a multi-dimensional and multi-disciplinary approach. More detailed guidance at a national level and the development of structured education could be steps towards improving clinical care and outcomes.

DMD Care UK

DMD Care UK (www.dmdcareuk.org) is a collaborative project between patient organisation (Duchenne UK, Duchenne Research Fund) and clinicians in the UK. The aim of the project is to agree and implement standards of care UK wide in DMD based on the 2018 international consensus. Expert working groups in each aspect of care are developed which include patient representatives. Recommendations for care delivery UK wide for endocrine and bone monitoring/management were drafted with wide consultation and approved in 2020 with endorsement by the British Society for Paediatric Endocrinology and Diabetes.



Initiatives (in endocrine/bone) developed by DMD Care UK and endorsed by the British Society for Paediatric Endocrinology and Diabetes.

