

## INTRODUCTION AND AIMS

Bone and endocrine complications are common in boys with Duchenne muscular dystrophy (DMD), especially those on long term steroid.

In 2020, a UK wide collaborative project between clinicians and patient organization (Funding Duchenne UK) called **DMD Care UK** [[www.dmdcareuk.org](http://www.dmdcareuk.org)] was launched. The project aims to facilitate implementation of standards of care in the UK, adapting recommendations from the 2018 international care consensus. The recommended endocrine/bone standards were used to audit practise in one UK tertiary centre.



The endocrine/bone standards are that all boys with DMD:

- Should be on vitamin D supplementation.
- Should have vitamin D levels checked every 12 months.
- Should have lateral spine imaging to screen for vertebral fracture every 12 months
- On steroid therapy should have access to hydrocortisone for injection at home with severe acute and/or vomiting illness
- Should have pubertal assessment if aged 12 years or older.

## INCLUSION AND EXCLUSION CRITERIA

We included all boys with DMD managed by the neuromuscular team at the Royal Hospital Children Glasgow in 2019. Patients included in the VISION-DMD clinical trial were excluded as it is unknown whether they are on steroids or the investigative drug (Valmorolone).

## METHODS

DMD patients managed at the Royal Hospital for Children were identified from a department database. Digital medical notes were accessed for all relevant data and recorded in a standardized proforma.

Results were expressed as median (range).

## RESULTS

49 patients were included in our study. Median age at last clinic visit in 2019 was 11.5 years (3.0, 17.3). 21/49 (43%) were older than 12 years. 37/49 (67%) were non-ambulant. 41/49 (85%) were on steroids- 3 had discontinued steroids, and 4 were diagnosed in 2019 and commenced steroids in 2019/2020.

### Vitamin D supplementation

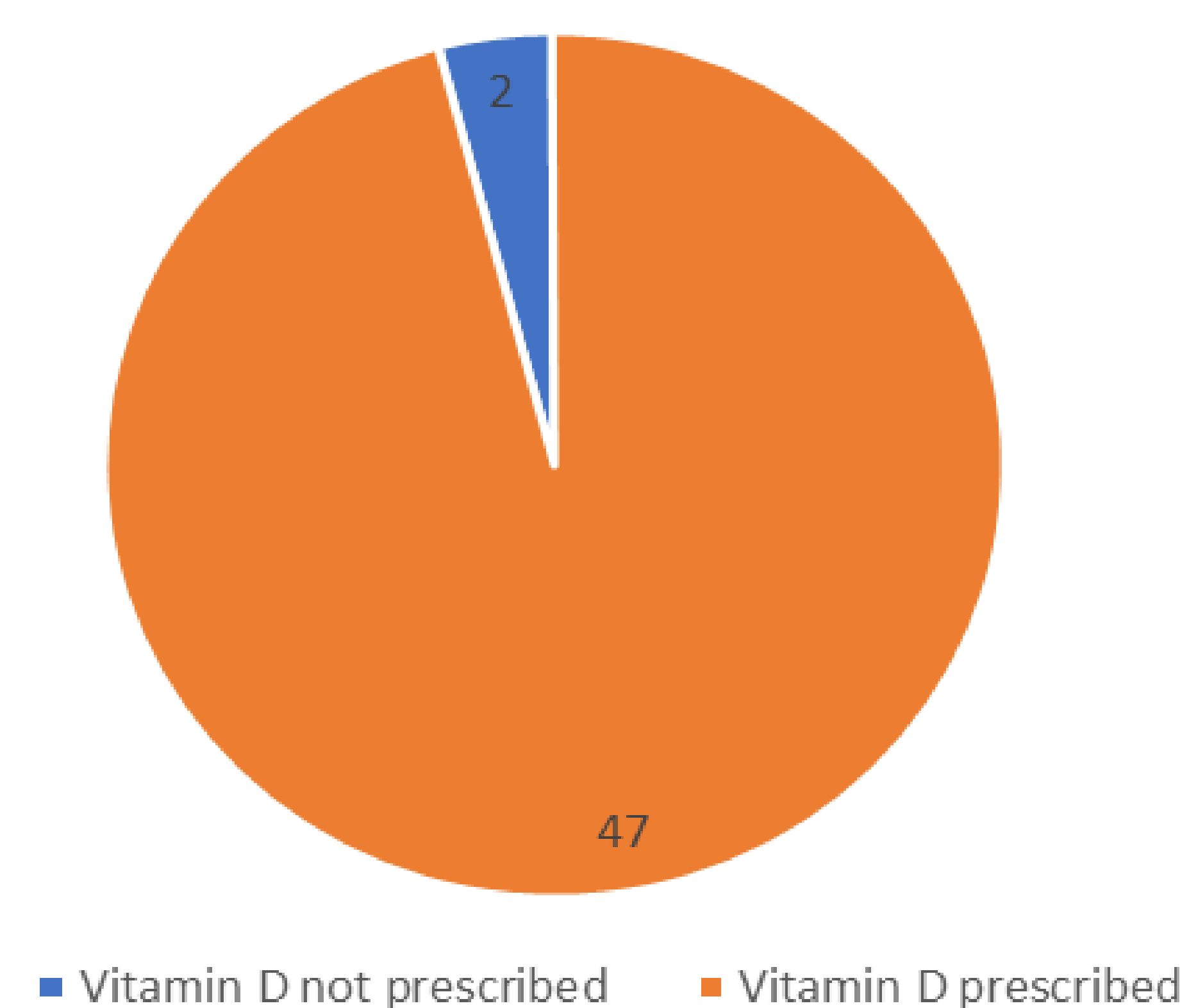


Figure 1: Vitamin D supplementation.

**The majority of boys (47/49-96%) with DMD were on Vitamin D supplementation.**

### Vitamin D monitoring

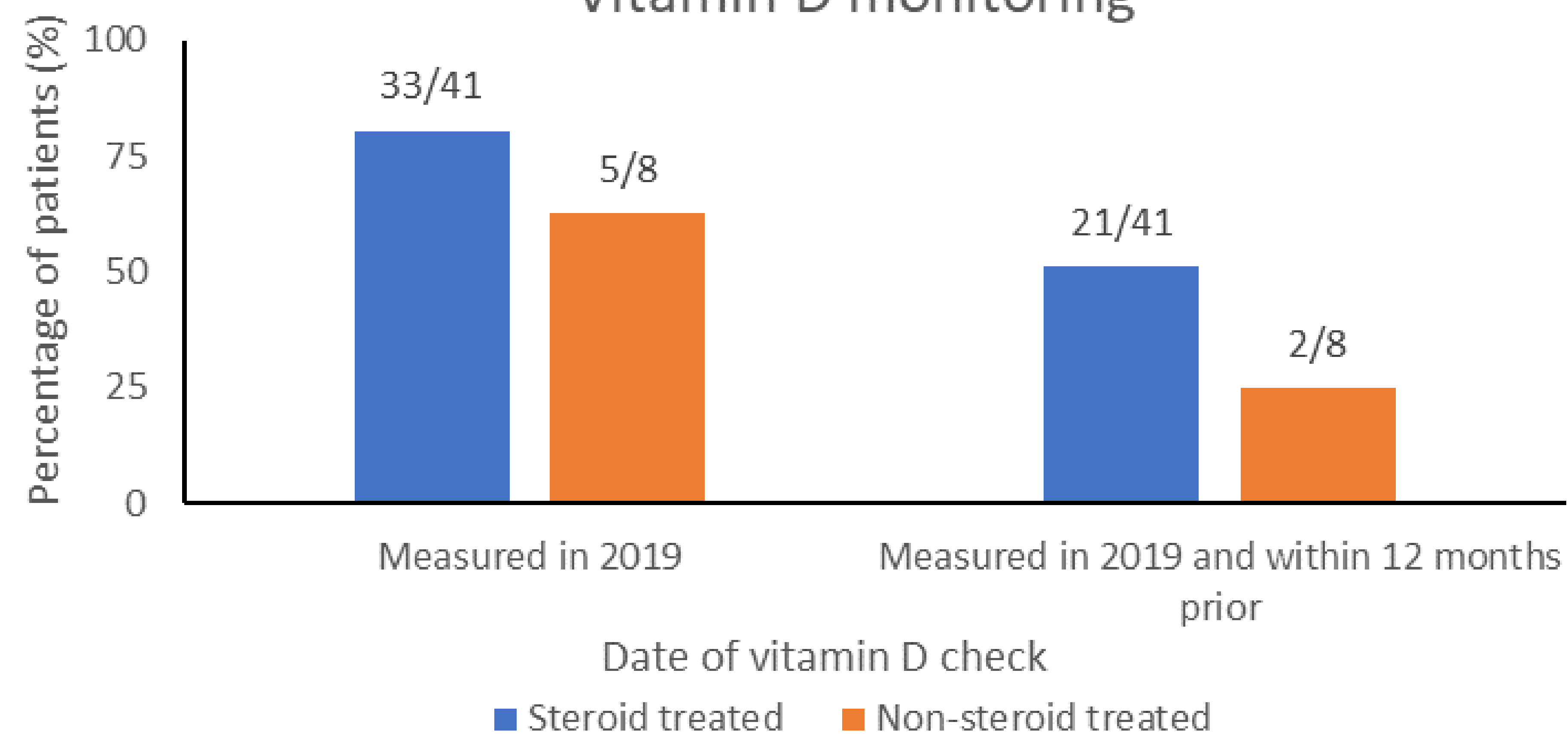


Figure 2: Monitoring of 25 hydroxy-vitamin D levels

**A large proportion of boys (38/49 -78%) in the total cohort had a measurement of 25-hydroxyvitamin D level checked in 2019. Over half (23/38-61%) of those who had a measurement of 25-hydroxyvitamin D level checked in 2019 had a previous measurement in the last 12 months (within a 365 days period).**

### Lateral thoracolumbar X-ray completion

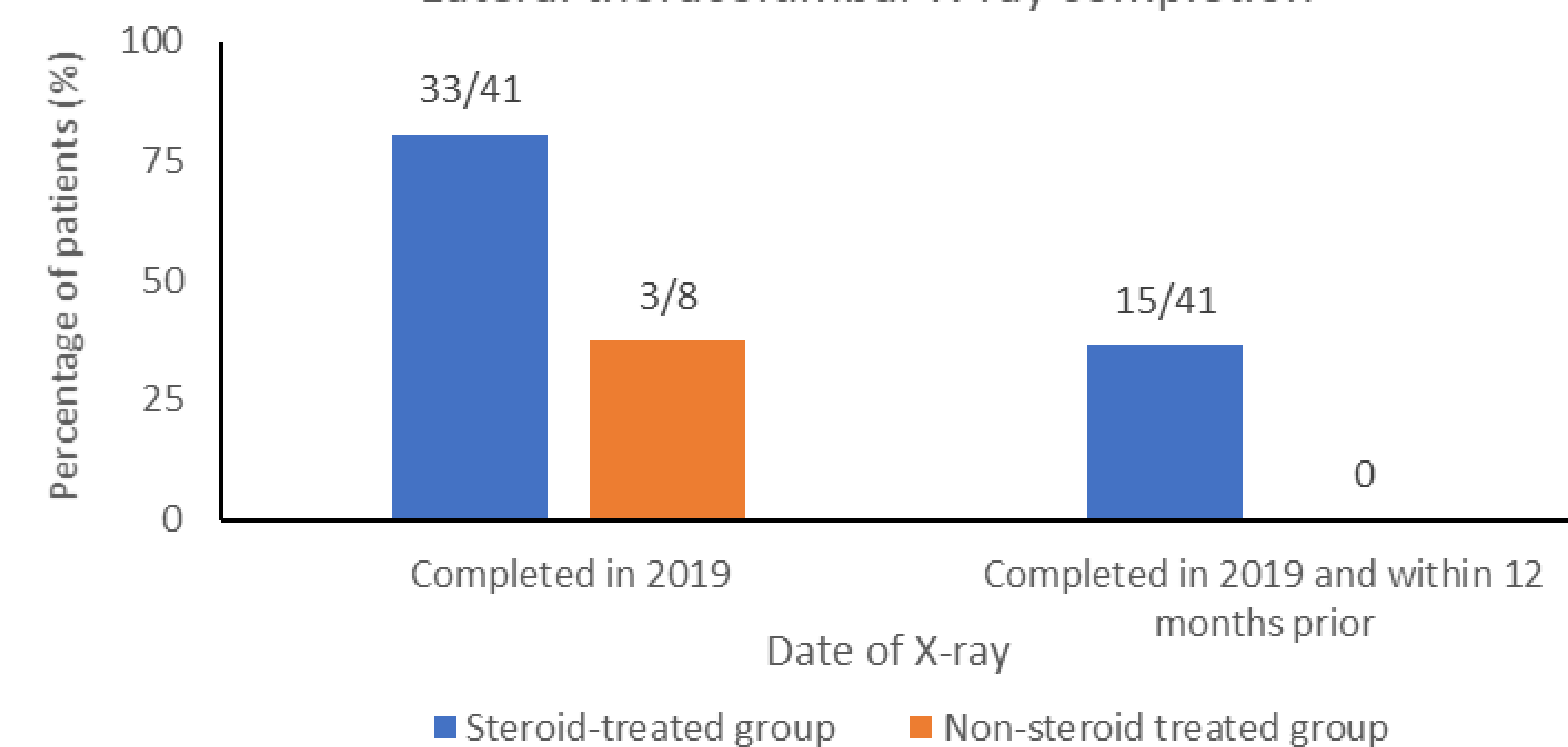


Figure 3: Lateral spine monitoring for vertebral fracture

**A large proportion of boys (36/49-74%) had lateral spine monitoring for vertebral fracture in 2019. However, under half (15/33-46%) of those who had lateral spine monitoring in 2019 had a previous imaging within the last 12 months (with a 365 days period).**

### IM hydrocortisone supply for steroid-treated patients

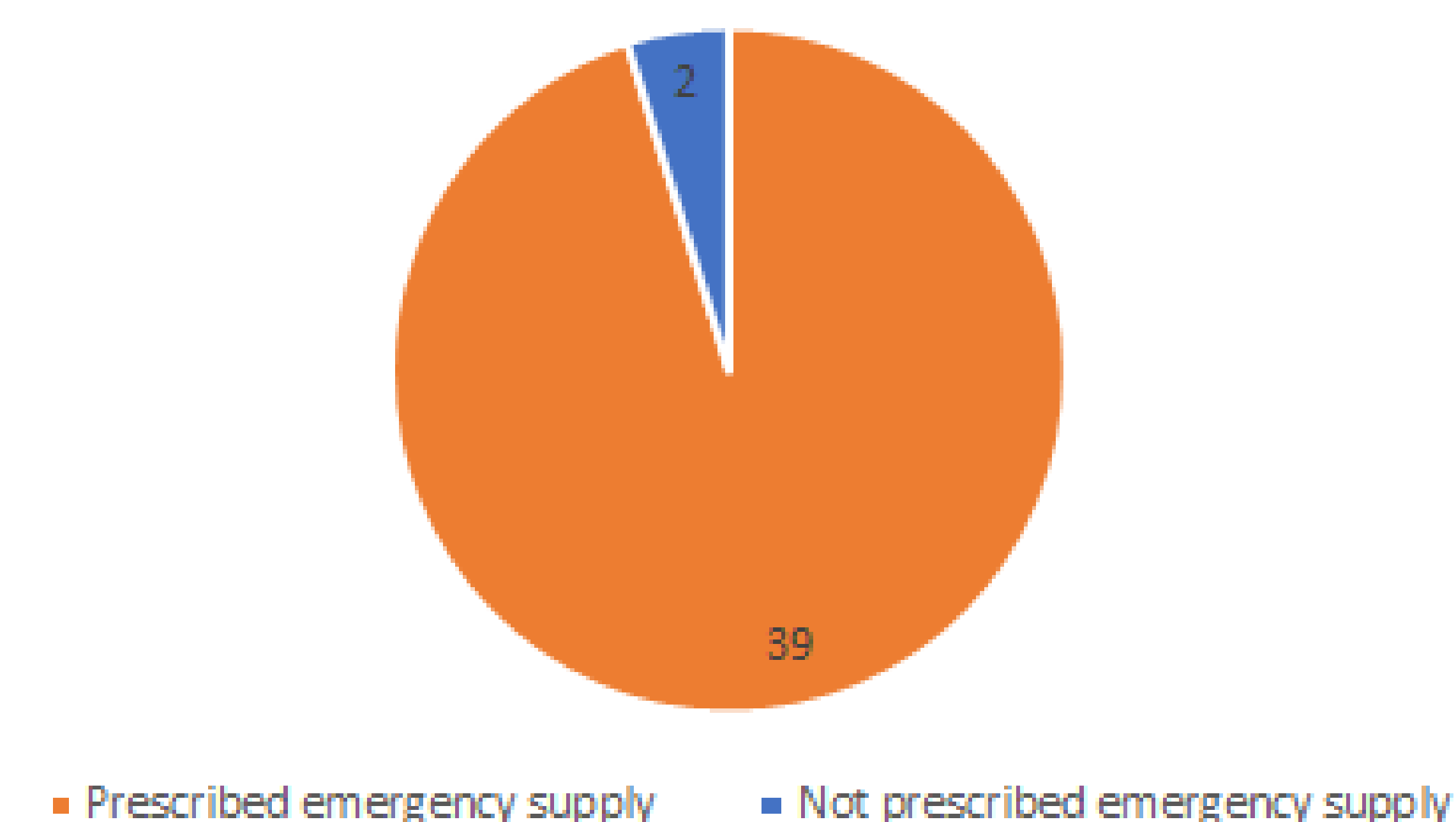


Figure 4: Access to home IM hydrocortisone in steroid treated boys.

**The vast majority (39/41- 96%) of steroid treated boys had access to IM hydrocortisone for use during severe emergency out of hospital. All those families also received training on IM injections. All 21 boys who were 12 years or older had clinical assessment of puberty in 2019.**

## CONCLUSION

This single-centre audit of endocrine and bone monitoring in DMD showed variable degree of adherence to current international and national guidance. Audit of other UK centres are planned and ongoing. Audit target and standards will be determined to set the bench mark for UK wide audit. Such plans are in discussion but require buy-in from numerous stakeholders.

