RECOMMENDATION OF ENDOCRINE & BONE MONITORING IN DUCHENNE MUSCULAR DYSTROPHY (DMD) IN THE UK NORTHSTAR NETWORK

This UK NorthStar guidance on bone and endocrine monitoring of boys with DMD for use by neuromuscular clinicians in the clinic is adapted from recommendations laid out in the International Care Consensus of management of DMD (2018).

BONE

- Annual screening lateral thoracolumbar spine imaging and DXA for bone mineral density in all boys is recommended.
  - Either lateral thoracolumbar spine x-ray or DXA based vertebral fracture assessment can be used.
- Prescribe Vitamin D supplements to all boys with DMD.
  - Blood monitoring of 25 hydroxy-vitamin D recommended annually in all boys with DMD aiming for at least 50 nmol/L.
- Refer or discuss with endocrinology/bone specialist for consideration of the use of bone protective therapy (e.g. bisphosphonate) following:
  - Vertebral fracture with or without back pain.
  - Low trauma long bone fracture.

PUBERTY

- Initiate conversation regarding likely pubertal delay and need for testosterone treatment in those treated with steroids during neuromuscular clinic follow-up at around 10-11 years of age.
- Assess puberty in all boys aged 12 years and older.
  - Formal assessment of pubertal status should be performed by a clinician with experience in assessment of puberty.
  - Refer boys with no signs of puberty to the endocrine clinic for discussion about testosterone therapy (ideally before 14 years). The testosterone replacement guidance for management of boys with hypogonadism developed by the British Society for Paediatric Endocrinology and Diabetes can be used.

ADRENAL

- All boys prescribed Prednisolone or Deflazacort regardless of regimen (daily or intermittent) should be assumed to have adrenal suppression and therefore at risk of adrenal crisis during acute illness or significant stress (eg surgery, bisphosphonate infusion, especially with first infusion).
- All boys prescribed steroid therapy should have written documentation of emergency steroid plan to be followed during acute illness.
- All families and boys should be educated about stress dosing plans and signs/symptoms of adrenal crisis.
- Wearing of medical alert bracelet and carrying of a steroid card is strongly encouraged especially for adolescent boys and adults with DMD.

FOR MILD-MODERATE ILLNESS WITH FEVER

- A plan for stress dosing with oral steroid should be in place during mild-moderate illness with fever (no vomiting), regardless of steroid regimen (continuous or intermittent).
- Consider stress dosing with steroids for bisphosphonate infusion, especially first infusion but can also occasionally be an issue with subsequent infusions.

(a) If using **Hydrocortisone** as oral stress dose, give this four times a day for at least 2 days [Table 1]. The usual *prescribed* steroid (Prednisolone or Deflazacort) should continue.

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(b) If using **Prednisolone** as oral stress dose, give this two times a day for at least 2 days [Table 1].
If taking a Deflazacort steroid regimen, the usual *prescribed* Deflazacort should continue.
If usual *prescribed* Prednisolone dose is higher than the suggested Prednisolone *stress* dose, split the *prescribed* Prednisolone and give in two doses (morning and evening).

- For example, *stress* dose Prednisolone is 5 mg BD, *prescribed* Prednisolone dose is 20 mg daily. In this case, simply split the usual *prescribed* Prednisolone dose and give Prednisolone 10 mg (morning) and Prednisolone 10 mg (evening).

Table 1: *Suggested oral stress dosing of steroids for mild-moderate illness with fever (no vomiting)*

	Stress Dose Steroid	
Weight (kg)	**Hydrocortisone**	**Prednisolone**
10 to 15	2.5 mg QDS	1.0 mg BD
16 to 35	5 mg QDS	2.5 mg BD
36 to 60	7.5 mg QDS	5 mg BD
61 to 100	10 mg QDS	10 mg BD

The above is an example of a dosing plan for ease of implementation in the neuromuscular clinic. Detailed local regimen should be followed if already in place.
If prescribed Prednisolone is higher than the suggested stress dose, split the prescribed Prednisolone and give in two doses (morning and evening). There is insufficient experience with the use of Deflazacort for oral stress dosing. Local endocrine guidance on frequency of administration can be followed if there is experience.

FOR SEVERE ILLNESS OR VOMITING ILLNESS

- Provide boys prescribed Prednisolone or Deflazacort regardless of regimen (daily or intermittent) a prescription for hydrocortisone by injection for emergency administration. The emergency injection could be administered by the family or the attending emergency medical services.

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Reviewed and endorsed by the Clinical Committee of the British Society for Paediatric Endocrinology and Diabetes.

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