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Research Report

Endocrine and Bone Monitoring in Boys with Duchenne Muscular Dystrophy; Do we adhere to the standards of care?

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In 2020, a national initiative funded by patient groups (Duchenne UK, Joining Jack and Duchenne Research Funds): DMD Care UK (www.dmdcareuk.org), was set up in collaboration with clinicians and academics within the UK NorthStar Network. The main aim of the DMD Care UK project is to agree upon and implement standards of care in the UK based on the 2018 guidance. Based on the UK recommendations, a two centre audit (Glasgow and Leeds) was performed of boys with Duchenne Muscular Dystrophy (DMD), managed in both sites during 2019. The audit aimed to assess the adherence of both centres to the new UK standards of care, aiming to highlight any areas for improvement, which could lead to improved endocrine and bone monitoring in this cohort. In addition, the audit would prompt further work into developing targets for the standards of care that could be applied nationally.

Experts in the bone-endocrine working group of DMD Care UK developed minimum UK standards in the area of bone-endocrine monitoring and management. Following consultation with all relevant stakeholders in the multi-professional UK NorthStar network, members of the British Paediatric and Adolescent Bone Group, the clinical guidance was formally endorsed by the British Society for Paediatric Endocrinology and Diabetes

The standards selected for this audit are as follows:

1. All boys with DMD should be on vitamin D supplements.
2. All boys with DMD should have annual measurement of 25 hydroxy-vitamin D levels.
3. All boys with DMD should have annual lateral thoracolumbar spine imaging to identify vertebral fracture (VF)
4. Clinical examination of puberty should be undertaken in all boys with DMD of 12 years and older
5. All boys on steroid therapy should have access to hydrocortisone for use as injection, with severe illness.

All boys (104 in total) with DMD under the clinical care of Leeds and Glasgow in 2019 were included in the analysis. 83/104 (80%) were on vitamin D supplements. 80/104 (77%) had measurement of 25 hydroxy-vitamin D levels in 2019 whereas this was only measured in 56/104 (54%) boys in 2018. 71/104

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Table 1

	Centre one		Centre two		Combined	
Total patients	55		49		104	
Mean age	10.51 years		10.9 years		10.7 years	
On steroids	43/55 (78%)		41/49 (84%)		84/104 (81%)	
1. All boys with DMD should be on vitamin D supplements.	36/55 (66%)		47/49 (96%)		83/104 (80%)	
	2018	2019	2018	2019	2018	2019
2. All boys with DMD should have annual measurement of 25 hydroxy-vitamin D levels.	33/55 (60%)	42/55 (76%)	23/49 (47%)	38/49 ((78%)	56/104 (54%)	80/104 (77%)
3. All boys with DMD should have annual lateral thoracolumbar spine imaging to identify vertebral fracture (VF)	17/55 (31%)	35/55 (64%)	15/49 (31%)	36/49 (73%)	32/104 (31%)	71/104 (68%)
4. Clinical examination of puberty should be undertaken in all boys with DMD of 12 years and older	11/23 (48%)		21/21 (100%)		32/44 (73%)	
5. All boys on steroid therapy should have access to hydrocortisone for use as injection, with severe illness.	38/43 (88%)		40/41 (98%)		78/84 (93%)	

(68%) had lateral thoracolumbar spine imaging for VF in 2019, whereas this was only performed in 32/104 (31%) boys in 2018. Of eligible adolescent boys, 32/44 (73%) had assessment of puberty in 2019. Of the 84 boys on corticosteroid therapy, 78 (93%) had access to emergency hydrocortisone for injection with severe illness. See Table 1 for results.

This audit of two tertiary neuromuscular centres in the UK showed variable adherence to the audit standards in the area of bone-endocrine care for boys with DMD, despite the 2018 international recommendations. However, we were able to document increasing numbers in standards two and three. This suggests that centres recognise the importance of these standards and that improvement is possible.

Managing the complex needs of boys with Duchenne muscular dystrophy (DMD) requires a multi-disciplinary approach. This is highlighted in the international standards of care updated in 2018 [1, 2]. The guidance was updated with respect to cardiac, respiratory, gastrointestinal, bone and endocrine management. However, implementing complex care requires consideration of national health systems and local resources. Several factors are likely contributing to failure to implement bone and endocrine standards. This could include patients not attending clinics or blood test appointments, as well as clinical teams finding it hard to deliver all aspects of the annual review of DMD patients. To improve this we would suggests using a standardised clinic proforma or check list. This could be completed by other members of the MDT such as the clinical nurse specialist prior to clinical assessment.

Wider scale audit following emergence from the COVID19 pandemic and with the 2020 UK specific guidance produced through DMD Care UK should now be conducted to evaluate impact on clinical care at a national level. The audit standards included in our current study can be used as key performance index to evaluate the quality of care. Determining standards of adherence to such key performance index will also allow bench-marking of clinical services, and should be developed for all aspects of clinical care of boys with DMD. This is essential to improve understanding and management of the endocrine complications of DMD such as osteoporosis and related fractures, impaired growth, delayed puberty and adrenal insufficiency.

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