AUDIT OF BONE HEALTH IN DUCHENNE MUSCULAR DYSTROPHY

Audit report completed February 2016

Contents page number

EXECUTIVE SUMMARY

Background:

Duchenne Muscular Dystrophy (DMD) affects about 1:3500 people (mainly boys) born each year in UK. It causes progressive muscle weakness. Typically by age 12 years people with DMD are wheel chair dependent.

Oral corticosteroids (OCS), if initiated between the ages of 4 and 8 years appear to slow the progression in muscle weakness and have become routine treatment for DMD.

Reduced exercise with weight bearing combined with the side-effects of OCS result in bone thinning and increased risk of fractures – especially of the vertebrae.

Audit:

This audit was carried out to determine whether children and young people in N Ireland with DMD were receiving a bone health management program that included the monitoring and active treatment of bone health issues.

We used internationally accepted guidelines for the management of DMD.

This case note audit determined whether monitoring (of blood bone biochemistry and by DEXA scanning at baseline and follow up), pre-emptive treatment (diet, Vitamin D, calcium supplementation and bisphosphonate therapy) and active treatments for fractures were being implemented.

Findings:

1) OCS are being started in a timely fashion and in all people (when indicated and tolerated).
2) DEXA scanning, but not necessarily bone biochemical profiling including Vitamin D levels, is routinely done at baseline and on follow up. Vitamin D levels have not regularly been tested but recent evidence suggests that this is now considered and carried out in all patients.
3) The neurology team rather than specialist dietitians appear to be giving the dietetic bone health advice for the majority. Endocrinology/bone specialists are only occasionally and in specific circumstances consulted.
4) Vitamin D and calcium supplementation appears to be started in a timely fashion.

There was evidence that this is now being initiated even prior to the starting of OCS.

5) Bisphosphonates (BPs) are increasingly being used.
6) There was evidence of undue delay in patients complaining of back pain and other symptoms in keeping with a fracture in receiving assessment and treatment.

Recommendations and Action Points

a) The GAIN audit "Standards of Care guidelines for people with Duchenne's Muscular Dystrophy in Northern Ireland" should have bone health as one of its core pathways (currently not the case).

b) Develop the bone health pathway for the above standards.

c) Convene meetings with management (Belfast HSC Trust), current multidisciplinary teams involved, and Endocrinology and Dietetics to agree:
   i) best ways to deliver an expert bone health pathway for people with DMD.
   ii) what resources are required for implementation?

d) Education of the multidisciplinary team regarding their roles and responsibilities concerning how acute bone issues should be managed and referred when a patient complains of a potential bone symptom.

BACKGROUND / RATIONALE

Duchenne Muscular Dystrophy (DMD) affects about 1 in 3500 boys born in the UK and is an inherited condition (X-linked, therefore mainly occurs in boys) affecting the proximal muscles causing muscle weakness. Those affected have a genetic abnormality that causes a reduced level of the dystrophin protein that is essential for muscles to function correctly. The lack of dystrophin causes muscle damage and inflammation leading to eventual fibrosis and muscle weakness.

The weakness is often first identified when the child is a toddler and has difficulty standing up / walking and progresses steadily. Without treatment an affected boy will lose ambulation by the age of 10-12 years and will eventually require the use of a wheel chair.

Due to postural imbalance and reduced muscular support to the spine scoliosis (curvature of the spine) typically develops. Significant scoliosis will be associated with progressive restriction of breathing and, most importantly, will make sitting in a wheelchair uncomfortable. Major corrective surgery is then required.

Although there is currently no cure, advances in treatments over the last two decades have resulted in increased survival for those with DMD. The anti-inflammatory effects of oral corticosteroids (OCS) have been used to slow the ongoing damage to muscle cells. Commencing OCS early (at age 5-6 years) can preserve ambulation up to age 15 years (and even longer) with much less likelihood of the development of scoliosis. Non-invasive respiratory support along with cough assist technology has helped address many of the breathing problems faced in later stages of DMD. The heart muscle is affected in DMD and eventually leads to cardiac failure of variable extent. Proactive prescription of ACE inhibitors and beta blockers has helped to prevent progression of heart failure.

However, an unwanted effect of oral corticosteroid (OCS) therapy is bone thinning (steroid-induced osteoporosis). This combined with the reduction in exercise and weight bearing result in people with DMD being more prone to fractures. A long bone or spinal fracture is a most unwanted complication and prevention or reduction of fracture risk is possible (guideline 2010[1]). In addition, low Vitamin D and Calcium levels are associated with poor bone structure.

Attention must therefore be given to supporting bone structure. In the past, treatment strategies for bone health were often reactive after a bone fracture rather than proactively preventing fractures.

Prevention of fractures is now seen as a priority and an indicator of good practice. Thus the early identification of osteoporosis and its treatment is an important part of a preventative strategy.

There are internationally accepted management guidelines ("Diagnosis and management of Duchenne muscular dystrophy, parts 1 & 2")[1] published in the Lancet in early 2010. These describe an optimal standard of care for people with DMD and have been endorsed by patient support groups such as Treat-NMD and by the patient group in Northern Ireland.

At national level, the North Star Clinical Network performed an audit (2010), involving seventeen mainland UK sites[2]. It was found that 58% of people with DMD were deficient in Vitamin D levels (15% had severe deficiency).

Preventative therapies (for osteoporosis) include monitoring for abnormalities in Vitamin D and calcium metabolism and for the presence of osteoporosis e.g. with DEXA or USS scans.

These are followed by **active treatment** with Vitamin D replenishment and prescription of bisphosphonates used to strengthen bone. It has been speculated that the use of OCS combined with bisphosphonates may slow the rate of deterioration of muscular weakness compared with steroid monotherapy.

Prior to this audit it was not known whether people with DMD in Northern Ireland are currently being offered adequate preventative monitoring and active treatment to maintain bone health and reduce the likelihood of fractures.

PROJECT TEAM

Member	Job/Speciality	Regional Trust
Sandya Tirupathi	Consultant Paediatric Neurologist	BHSCT
Isobel Douglas	Nurse Specialist	BHSCT
Mark Elliot	Consultant Radiologist	BHSCT
Michael Shields	Consultant Paediatrician	BHSCT
Alison Livingstone	Community Paediatrician	NHSCT
Bronagh Blackwood	Senior Lecturer	Queen's University Belfast
Jennifer Bell	Research Fellow	Queen's University Belfast

AIMS / OBJECTIVES

The overall aim of this audit was to determine whether the service proved to children with DMD meet the required standards for bone protection in accordance with best practice.

STANDARDS

We based our assessments on the following international standards:

- Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Bushby et al. (2010).[1]
- North Star Medical Assessment Sheet, North Star Clinical Network for paediatric neuromuscular disease[2].
- 170th ENMC International Workshop: Bone protection for corticosteroid treated Duchenne muscular dystrophy 2009 Naarden, The Netherlands. R. Quinlivan et al. (2010)[3].

Using the above, a focused set of standards were agreed by the Bone Health in DMD Audit Steering Group (Appendix 1).

Specific standards addressed in this audit.

A} <u>Monitoring</u>:

At baseline: (typically this is age 3+ years and/or at start of OCS therapy)

1. In Serum: Calcium, Phosphate, Alkaline phosphatase, Magnesium, PTH (parathyroid hormone) level (**Biochemical bone profile**), 25-OH **Vitamin D** level.

2. DEXA (dual-energy x-ray) scan

At follow up:

For those on long term OCS or at known extra fracture risk

1. Biochemical bone profile, Vitamin D (annual)
2. DEXA scan – two yearly unless previous DEXA scan showed significant osteoporosis (*Z* score <–2).

When a bone problem has been identified e.g. reported back pain:

If back pain is present and /or if kyphoscoliosis is noted on clinical examination therapy

- Spine radiograph on awareness of problems– WITHIN 2 WEEKS (Possible presence of vertebral compression fracture).

B} <u>Interventions</u>: on awareness of problems– WITHIN 2 WEEKS

- Vitamin D
    - Vitamin D treatment/replenishment for proven deficiency (serum 25 hydroxyvitamin D < 50 mg/ ml) is necessary
    - Supplementation should be considered in all children if levels cannot be maintained
- Calcium
    - Calcium intake and possible supplementation should be carried out in consultation with a dietician

- Bisphosphonates
    - Intravenous bisphosphonates are indicated for vertebral fracture
    - Oral bisphosphonates remain controversial as treatment or as a prophylactic measure but are generally recommended.

C} Referrals:

- To paediatric endocrinologist/bone specialist if bone-age measurements are abnormal (>2 SD below the mean) – the urgency of appointment depends on the clinical problem
- Orthopaedics –
    - when there is suspicion of a fracture assessment for use of intravenous bisphosphonates is urgent
    - the development of scoliosis needs orthopaedic assessment but this is not urgent and has not been included in this audit.

METHODOLOGY

This N Ireland regional audit collected data retrospectively from the clinical records of those children 3 years and over with a confirmed diagnosis of DMD.

Sample

- All people (3+ years) with confirmed diagnosis of DMD alive and living in Northern Ireland, attending the specialist DMD neuromuscular clinic.
- Study period: May 2014 - June 2014.
- Clinical case notes, prescription records, biochemistry (laboratory) and radiology imaging results along with individual notes kept by the Neuromuscular Support Nurse belonging to those attending the DMD clinic were used to inform the audit.

Audit tool / Data collection

- Audit proforma approved by GAIN and the Bone Health in DMD steering group (Appendix 1).

- An initial pilot was carried out with the research Fellow (JMB) and Consultant Paediatric Neurologist (ST) in the BHSCT. Once the audit proforma was optimised the research Fellow collected the data for the study period.

Data analysis

- The research Fellow carried out the initial data analysis and prepared a first draft report.
- Clinical experts were consulted for clarification on the clinical relevance of data.
- Members of the Steering Group reviewed the audit report and suggested changes with an action plan and recommendations.

FINDINGS AND OBSERVATIONS

In this audit the clinical records of 56 people with DMD (aged 3 to 33 years) who attended a regional Neuromuscular Clinic in Belfast were included; 54 were male (96%). Thirty two (57%) were walking independently (indicating that that most will be in the childhood and young person age range), and 38 (68%) were taking oral corticosteroids during the audit period.

To address the audit questions and put into local context we drew up some key questions to answer. The results (findings/observations) are reported under each question. We had to do this because the core questions in this audit of bone health in DMD depended on whether the people with DMD had been offered OCS in the first instance (which is strongly recommended good practice).

The recommended age for initiation of OCS is 4-8 years when the maximal natural muscle function increase has occurred and before deterioration starts. OCS should be given at least until people with DMD are wheelchair dependent and have significant loss of muscle power. It is less clear whether OCS should be continued for longer term after this has occurred e.g. after 15 + years. In addition, it is also less clear whether the benefits from initiation of OCS extend to older patients who already are wheelchair dependent and have suffered major loss of muscle function already. Overall in recent years, OCS are being used for longer and have been initiated in older patients especially in those not experiencing OCS side-effects. In this audit whilst we studied all N Ireland people with DMD (aged 3+ years) we have also concentrated our efforts on studying children aged 5-10 years and 11-15 years of age. This reflects our more recent practice and represents a population in which the current guidelines definitely apply.

KEY QUESTIONS and AUDIT FINDINGS/RESULTS

1. What is the current status of bone health in people with DMD in Northern Ireland?

Current status of bone health from the audit cross-sectional snapshot

In those individuals with DMD (n=56):

- 22 (39%) have a spinal deformity (mostly scoliosis),
- 12 (21%) recently complained of bone pain,
- 12 (21%) had suffered a long bone fracture,
- 1 (2%) had suffered a vertebral fracture,
- Bone Mineral Density DEXA scanning: median corrected z-score was -1.80.

2. Does the age at which OCS treatment is first started in people with DMD compare to that recommended? Have all people currently 5-10 years of age started receiving OCS treatment?

There are no recommendations given in guidelines with respect to the age for commencing OCS treatment as this is disease dependent. OCS are usually started when the patient's motor skills reach a plateau before decline at around the age of 4-8 years. Given that the routine use of OCS has only recently become standard practice, some older children therefore were first started on OCS at an older age.

We therefore specifically looked at the group currently aged between 5-10 years to reflect our current or recent practice.

- 19 of the 21 children currently aged between 5-10 years were prescribed OCS. Of the two not on OCS one was a female (atypical case) and the second had a comorbid condition making OCS relatively contraindicated.
- All boys with DMD currently aged between 5-10 years were being treated with, or had been tried on, OCS when this had been deemed appropriate.
- For children currently aged between 5-10 years the median age for starting OCS treatment was 6 years (IQR 6-7 years, range 5-8). This is comparable to rates reported in the rest of the UK and in keeping with best practice.

3. **Are baseline bone health assessments being carried out at the start of OCS treatment? Baseline assessments allow early detection of deterioration after treatment with OCS and with disease progression.**

- The youngest individual who had a DEXA scan was 4 years of age (guideline recommended age 3+ years).
- 16 of the 19 (84%) children currently aged between 5-10 years and who were taking OCS had a baseline DEXA scan prior to or at the time OCS were started. The median age was 6 years at the time of 1st scan.
- Three children did not have a DEXA scan when starting OCS treatment. Reasons why one of these children did not have a DEXA scan related to behavioural issues around allowing satisfactory scanning. One of the three children did have a long-bone fracture 2 years after starting OCS treatment.
- Only 4 of 19 (21%) children currently aged 5 -10 years and taking OCS had serum vitamin D and a bone biochemical profile measured before taking OCS.

4. **Do all people taking OCS have regular bone health assessments?**

- Given that many children (aged between 5-10 years) initiated on OCS were not old enough to have had a repeat DEXA scan we looked at those aged 11-15 years.
- All 8 young people with DMD currently aged 11-15 years taking OCS had a DEXA scan at a median time of 1.5 years after starting OCS (maximum time delay until repeat DEXA scan was 3.5 years).
- Generally an attempt to perform a DEXA scan was made in all patients at least every 2 years (range 1-4 years).

5. Was monitoring of Vitamin D levels and bone biochemical profiling completed?

Vitamin D and bone biochemical profile monitoring was NOT routinely checked annually. In those currently aged between 5-10 years 53% and 63% had Vitamin D and bone profiles measured. In those currently aged between 11-15 years 79%

and 88% had Vitamin D and bone profiles measured. In those currently aged between 16-19 years 84% and 100% had Vitamin D and bone profiles measured.

6. Are all people with DMD offered bone health interventions when indicated in a timely and standardised fashion to optimise bone health?

The researcher (JMB) found that a bone protection strategy was considered in all and was generally commenced when people with DMD were started on oral OCS treatment. This involves vitamin D supplements, advice to increase calcium dietary content and baseline DEXA imaging to measure bone mineral density with repeat scans every 2 years.

In addition, there was recent evidence that a bone protection strategy was being considered even prior to the initiation of OCS. Thirty-seven percent (7 of 19, aged between 5-10 years and on OCS) had been started on vitamin D supplements before starting steroids.

The Neuromuscular clinic takes the responsibility to commence bone protection strategies to prevent steroid-induced osteoporosis. This service was found to operate mainly in isolation from bone, endocrine, and dietary specialists.

7. Are all people taking OCS treated with bone health interventions?

Interventions used for people with DMD in Northern Ireland included bisphosphonates (BPs), vitamin D and calcium supplements and physiotherapy.

The guidelines recommend; ***Vitamin D*** *treatment for proven deficiency and supplementation in all children if vitamin D levels cannot be maintained.* ***Calcium*** *intake and possible supplementation should be carried out in consultation with a dietician.* ***Intravenous bisphosphonates*** *should be used to treat a vertebral fracture.* ***Oral bisphosphonates*** *as treatment or as a prophylactic measure remain controversial but at the RBHSC and following practice at other UK neuromuscular centres these are prescribed to all people with DMD taking OCS and if DEXA z-score is <2.*

- Although advice was given to people and parents on increasing dietary calcium in all people starting OCS treatment, **a dietician was not involved in**

the process. There is limited availability of paediatric dietetic advice generally and there is no established pathway for referral for this category of patients. Also the initial vitamin D supplement used would have also provided supplemental calcium as it was a combination medicine providing both Vitamin D and calcium.

- The majority (>80%) of people were taking Vitamin D whilst taking OCS.
- Time of starting Vitamin D supplementation corresponded to the initiation of OCS treatment in 15 of 19 (79%) of people aged between 5-10 years. The median age of those between 5-10 years starting vitamin D supplementation was 6.5 years (IQR 6-8 years, range 4-9 years). Vitamin D was started at a median of 1.5 weeks after commencing OCS. Interestingly, thirty-seven percent (7 of 19) had been started on vitamin D supplements before starting steroids.
- Bisphosphonates were prescribed to 58% (22/38) of people taking OCS treatment. Of these 44% (10/23) people were prescribed BPs within 0.31 years (IQR -2.44-2.52, range 0.55-1.49) of having a bone mineral density z-score < 2.
- In children of 5-10 years of age, when OCS treatment was commenced, BPs (used in 63%, 12 of 19) were started at a median age of 8 years (IQR 6-8.5 range 5-9 years).

8. At what age are people started on bisphosphonates? How does this compare to recommended treatment management?

Oral BPs improve bone density in those with reduced bone mineral density with DEXA z-scores <-2.00 and are used widely for this purpose in people with DMD. Given that some recent evidence (weak) has come out that the combination of OCS and BPs may improve muscle function more than OCS alone there has been increased use of BPs and not just limited to when DEXA z-scores <-2.00

- 22 of 38 (58%) of people taking OCS treatment took oral BPs for bone protection, and one stopped BPs due to adverse effects (severe joint pain).
- An additional 4 people not taking OCS were taking BPs – they were older than 19 years of age and/or had a low DEXA scan z-score <2.

- Overall 26 (48%) of people with DMD (n=56) were taking BPs.
- Those currently aged between 5-10 years started BPs at a median age of 8 years (IQR 6-8.5, range 5-9 years) and this was a median of 1.69 years (IQR 1.05-2.81, range 0.0-12.94 years) after starting OCS treatment.
- A DEXA scan z-score of < -2 (IQR -2.50 to -2.02, range -3.40 to -1.50) was found in 20% (11/56) people, oral BPs were prescribed within a median of 16 weeks (-28.7 to 77.7, range -127.3 to 131.5 weeks). Five people were already taking BPs when their z-score diminished to below -2.
- The researcher noted that Bisphosphonate treatment has commenced earlier in children currently of 5-10 years of age and as the use of oral BPs has become more common they have now been given to older people and not just those who have DEXA scan z-scores below -2.

9. Are people with DMD assessed within two weeks when a problem or event associated with bone health occurs?

If people have suspected spinal deformity, bone pain or vertebral fractures spine radiographs should be carried out to investigate, within two weeks. This target, based on guideline suggestions and endorsed by the RBHSC, **was not met**.

- For suspected spinal deformity, 24 of 56 (43%) of all people with DMD had a spine radiograph within a median of 29 weeks (IQR 23.5-78.8, range 23.5 to 564 weeks) from clinical examination. Of these individuals 71% (17/24) were taking OCS. This delay likely represents the wait to be seen and assessed by orthopaedic surgery. We do not see this as an issue for this audit. Once an early scoliosis is identified children are referred to orthopaedics where they are typically monitored for the progress of the scoliosis so that timely surgical intervention can occur. The resources available to the orthopaedic service and its ability to provide this service were out with this audit scope.
- 2 of 9 (22%) of people reporting back pain were investigated with a spinal X-ray at a median of 19 weeks (IQR 16.7-21.4, range 14.6-23.5 weeks). The remainder did not have a spinal X-ray. [Case example: A vertebral fracture occurred in one patient taking OCS treatment at 9 years of age. The patient had back pain 17 weeks earlier. An X-ray was eventually taken when the patient was

admitted to hospital with severe back pain. This case illustrates problems caused by not having rapid investigations and, furthermore, IV pamidronate BP treatment was given but delayed until 9 weeks (65 days) after the person was admitted to hospital and when a vertebral fracture was identified.

10. Are people with DMD referred to Endocrinology and bone specialists?

It is recommended that management of potential bone health problems should be in conjunction with specialists in bone health and endocrine assessment.

- Only 6 of 56 (11%) of all people with DMD were referred to a paediatric endocrinologist. One patient had an abnormal bone-age measurement and the remainder had reduced measured height compared to standardised centiles for age. Thirteen percent (5/38) of people taking OCS treatment had a referral and 5% (1/18) people not taking OCS.
- 38 of 56 (68%) of people with DMD were referred to a bone specialist for spinal deformity, bone pain, fractures, or a combination of these. No referral was made within 2 weeks from a presentation of a bone health problem. The inappropriate delay mostly related to delayed diagnosis of cause of bone pain and for fractures. Scoliosis typically develops over a period of many months. The care service provided by Endocrinology and bone specialists needs to be specified in a Care pathway for lifelong care of people with DMD.
- At present the use of Endocrinology/bone specialist services is ‘as required’ and is not consistently/routinely offered in all people with DMD. That is, whilst children with DMD are regularly monitored by respiratory, cardiac and orthopaedic specialist services NO regular monitoring/treatment is provided by endocrinology and bone health services are currently provided by the Neurology service. Important to remember that the TreatNMD Guidelines came in 2010 and there was no pathway to endocrinology for these patients in N Ireland prior to this.

11. Are there other gaps in multidisciplinary care?

The research found the following gaps:

- Access to a specialist dietetic service. There is no traditional pathway and current no capacity to such a specialist dietetic service.
- Access to Endocrinology / bone specialist. There is no traditional pathway and current no capacity to such a specialist endocrinology/bone specialist service.
- Physiotherapy – specifically aimed at weight-bearing exercises. Physiotherapy has not been adequately funded for this category of patients

Summary of audit findings

AREAS OF GOOD PRACTICE

- Children are started on OCS in a timely fashion
- Those started on OCS are placed on a bone health protection regime with Vitamin D supplementation started early and there is increasing evidence of the earlier use of BPs.
- DEXA scan monitoring.

AREAS WHERE IMPROVEMENTS ARE NEEDED

- Biochemical bone profiles and Vitamin D levels at baseline and on regular follow up.
- Access to specific dietetic advice.
- Access to Endocrinology/bone specialists for monitoring and treatment.
- Delays occurred in assessing and treating potential fractures.

AREAS OF UNCERTAIN GOOD PRACTICE

- Given that most, but not all, children are now initiated onto OCS in a timely fashion – do those that are not on OCS get the same monitoring service? There were small numbers in this group (5-10 years) but the researcher found some evidence (data not presented) that this might not be the case.

RECOMMENDATIONS

a) Involve Endocrinology/Bone specialists in the routine monitoring and treatment of people with DMD.

b) Provide all people with DMD easy and regular access to expert dietetic service advice.

c) Create a bone health pathway stating the minimum baseline and follow up monitoring that is required. This should be part of the N Ireland DMD management guidelines aimed at standardising services and is currently under construction (GAIN: Standards of Care guidelines for people with Duchenne's Muscular Dystrophy in Northern Ireland).
Make sure bone health is part of this local guideline.

- A core set of clinically useful bone health assessments which can be conducted in all people with DMD as part of routine community or hospital care. For example vitamin D and biochemical bone profile could be measured yearly by patient's GP practice.

- Lifelong Bone health monitoring should be embedded in routine care - for example, automatically generated annual or biennial appointments for each patient for DEXA scanning and blood tests with a Practice nurse in primary care or at DMD clinics. It would be good practice to have the results of bone health assessments available before visits to the Neuromuscular clinic.

d) Provide education to all multidisciplinary teams regarding the urgency for assessment when a patient with DMD reports pain especially back pain. This also should be provided to people with DMD and their carers

ACTION POINTS

e) The GAIN audit "Standards of Care guidelines for people with Duchenne's Muscular Dystrophy in Northern Ireland" should have bone health as one of its core pathways (currently not the case).

f) Develop the bone health pathway for the above standards. Need to set up a team of endocrine/bone specialists (? Involve Sheffield Bone Unit – who visit Belfast as part of an outreach clinic)

g) Set up meetings with management (Belfast HSC Trust), current multidisciplinary teams involved, and endocrinology and dietetics to find out best ways to deliver an expert bone health pathway for people with DMD. In addition, to find out what resources are required to implement this.

h) Set up a multidisciplinary meeting/presentation to inform the multidisciplinary teams regarding how acute bone issues e.g. vertebral fractures are difficult to diagnose, how they present and what each team member should do if a patient complains of a potential bone symptom to them.

i) A member of the DMD Audit Project team should sit within the GAIN Guideline group which are currently being developed by GAIN

ACTION PLAN (PAGE1)

Audit Title _____

	Action *(i.e. How Recommendation will be implemented)*	Date Implementation to be completed	Staff Responsible	Manager Responsible	Change Stage (see key)	**Change Stage Key**
1	Involve Endocrinology/Bone specialists in the routine monitoring and treatment of people with DMD	6 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis, (Royal Belfast Hospital for Sick Children)	3	1. Agreed but not actioned
2	Provide all people with DMD easy and regular access to expert dietetic service advice	12 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis, (Royal Belfast Hospital for Sick Children)	1	2. Action in progress
3	Create a bone health pathway stating the minimum baseline and follow up monitoring that is required	12 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis, (Royal Belfast Hospital for Sick Children)	1	3. Partial implementation
4	A member of the DMD Audit Project team should sit within the GAIN Guideline group which are currently being developed by GAIN	3-6 months	I Douglas	Julie Lewis, (Royal Belfast Hospital for Sick Children)	1	4. Implementation complete

ACTION PLAN (PAGE 2)

Audit Title _____

	Action *(i.e. How Recommendation will be implemented)*	Date Implementation to be completed	Staff Responsible	Manager Responsible	Change Stage (see key)	**Change Stage Key**
5	The GAIN audit "Standards of Care guidelines for people with Duchenne's Muscular Dystrophy in Northern Ireland" to have bone health as one of its core pathways.	3 months	GAIN staff		1	1. Agreed but not actioned 2. Action in progress 3. Partial implementation 4. Implementation complete
6	Develop the bone health pathway for the above standards in consultation with relevant endocrine/bone specialists	12 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis (Royal Belfast Hospital for Sick Children)	1	
7	Meet with management teams in Belfast HSC Trust, current multidisciplinary teams, and endocrinology and dietetics to find out best ways to deliver an expert bone health pathway for people with DMD. Determine what resources are required to implement this.	12 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis (Royal Belfast Hospital for Sick Children)	1	

ACTION PLAN (PAGE 3)

Audit Title _____

	Action *(i.e. How Recommendation will be implemented)*	Date Implementation to be completed	Staff Responsible	Manager Responsible	Change Stage (see key)	**Change Stage Key**
8	Set up a multidisciplinary meeting/presentation to inform the multidisciplinary teams regarding how acute bone issues e.g. vertebral fractures present and should be managed. Provide education to MDT staff to enable them to urgently assess those with DMD presenting with back pain. Provide information to service users to empower them to seek help early	12 months	MD Shields, S Tirupathi, I Douglas	Julie Lewis (Royal Belfast Hospital for Sick Children)	**1**	1. Agreed but not actioned 2. Action in progress 3. Partial implementation 4. Implementation complete

Project Lead Signature:	**Name (printed) Michael D Shields**	**Date:18th Feb 2016**

In signing this, I agree the above action plan recommendations and, if necessary, will take a lead in ensuring that changes are made in order to obtain improvements in the quality of care

Managerial Lead Signature:	**Name (printed)**	**Date:**

REFERENCES

[1] Bushby *et al.* 2010. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol 2010; 9: 177–89.

[2] UK North Star neuromuscular clinical network (NSCN): national audit results in Duchenne Muscular Dystrophy (DMD) North Star Medical Assessment Sheet, North Star Clinical Network for paediatric neuromuscular disease. Neuromuscular Disorders 2010; Vol 20, Suppl 1, S8.

[3] Quinlivin R, Shaw N, Busby K. 170th ENMC international workshop: Bone protection for corticosteroid treated Duchenne Muscular Dystrophy. 27-29th November 2009, Naarden, The Netherlands. Neuromuscular Dosord 2010, Nov: 20(11): 761-9.

Data collection tool

AUDIT: **BONE HEALTH IN BOYS WITH DUCHENNE MUSCULAR DYSTROPHY**

Characteristics

Year of birth	
Date of DMD diagnosis	__/__/____
Does the patient require ventilation support?[a] (delete the non-applicable item)	1. Yes 2. No
Date at loss of independent walking[a]	__/__/____

Corticosteroid treatment[a]

Date when started steroids		__/__/____
Starting corticosteroid	**Drug**	1. Prednisolone 2. Deflazacort
	Dose	
	Patient Weight (kg)	
	Regime	1. Daily 2. Intermittent 3. Other _____
Current treatment	**Drug**	1. Prednisolone 2. Deflazacort
	Dose	
	Patient Weight (kg)	
	Regime	1. Daily 2. Intermittent 3. Other _____
Side-effects / complications		1. Yes – a)minor b) substantial 2. No

Bone health status[a]

Bone pain	1. Yes 2. No	Date				
		Location				
Fractures	1. Yes 2. No	Date				
		Location				
Vertebral	1. Yes	*Date noted*				

deformity	Kyphoscoliosis Scoliosis Other _____ 2. No	__ / __ / __ __ / __ / __ __ / __ / __

Before taking Steroids[b]

Test		**At 2 consecutive dates:**	
		Date	Level
Serum	**Ca**		
	Phosphate		
	Alkaline Phosphatase		
	25 OH Vit D		
	Mg		
	PTH level		
Urine	**Ca**		
	Na		
	Creatinine		

Imaging	**At 2 consecutive dates:**	
	Date	Level
DEXA – Lumbar spine – Bone density (g/cm)		1. Bone density (g/cm) _____ 2. z-score _____
		1. Bone density (g/cm) _____ 2. z-score _____

Radiograph	Spine		Deformity –
			Deformity –
	Left wrist		Bone-age –
			Bone-age –