Orthopaedic Guidelines on Management of Duchenne Muscular Dystrophy

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PART A: Principles of Orthopaedic Care in DMD

A1. Importance of orthopaedic care in DMD

Orthopaedic care is an area of particular priority for people with Duchenne muscular dystrophy (DMD) for a number of reasons.

- Widespread use of long-term corticosteroids along with reduced weight-bearing, has a negative impact on bone health and makes fractures more likely (Buckner et al., 2015; Ciancia et al., 2022; King et al., 2007).
- As skeletal muscles weaken, falls are a bigger risk, further increasing the chance of fractures.
- When still-ambulant individuals have a fracture, being immobilised for any significant period of time can significantly impact their ability to regain ambulation .
- The risks of fat embolism syndrome (FES) are higher than in people without DMD – even where there is no obvious fracture or significant trauma (Feder et al., 2017; Ida et al., 2018).

For these reasons, this DMD Care UK orthopaedic guideline has a significant focus on the **management of fractures** (see also Part B) in order to make sure that decisions taken are based on evidence and expert opinion and result in the best outcome possible for patients. Important **considerations for elective surgery** (see also Part C) are also presented.

A2. Key principles

Preserving mobility and independence by maintaining weight bearing status and / or wheelchair use is a priority in patients with DMD (Birnkrant, Bushby, Bann, Alman, et al., 2018).

Whilst elective orthopaedic care will be delivered at specialist orthopaedic centres, a patient with an acute injury is likely to present to their local trauma unit. Unnecessary patient transfers must be avoided, and appropriate fracture management may be delivered locally **if the necessary medical and anaesthetic expertise is available and as long as there is prior discussion with the regional specialist centre** (see guideline statements, below). We consider that direct communication between senior clinicians is key to safe management in this patient group.

Most patients with DMD take corticosteroid medication (prednisolone, deflazacort or vamorolone) which leads to bone fragility and increased fracture risk (Birnkrant, Bushby, Bann, Alman, et al., 2018; King et al., 2007).

Long term treatment with corticosteroids means that patients are at risk of adrenal insufficiency due to adrenal suppression (see Endocrine Guidelines: https://dmdcareuk.org/clinical-recommendations; (Birnkrant, Bushby, Bann, Apkon, et al., 2018)). Extra corticosteroids are needed when corticosteroid-treated patients with DMD present with a fracture. Extra corticosteroids, given intravenously (IV), are also needed during any surgical procedure in these patients.

Fat embolus syndrome (FES) is a particular risk for patients with DMD, with fatalities reported (Feder et al., 2017; Wihlborg et al., 2019). It has been reported in DMD even after minor trauma (Ida et al., 2018) and should always be considered as differential diagnosis particularly in a patient with DMD who develops acute respiratory symptoms, drowsiness, confusion, agitation and/or petechial rash after trauma or fracture (usually within the first 3 days). **Warning: the signs of fat embolism may be subtle.**

In the UK there is a well-established network of paediatric neuromuscular expert centres known as the North Star. These centres coordinate care for children with DMD and can advise specialist care (Appendix A). The adult North Star network has been established more recently and can help guide care for adult patients (Narayan et al., 2022).

All patients with DMD should be under the care of a specialist neuromuscular MDT with links to a specialist orthopaedic team when needed but regular review by an orthopaedic surgeon should not be necessary. Guidelines for physiotherapy and occupational therapy management are available for this patient group (https://tinyurl.com/5ha2uky6, accompanying paper in press).

In summary: DMD is a multi-systemic condition and patients' needs are often complex. The cardiac and respiratory involvement as well as the chronic treatment with corticosteroids and risk of adrenal crisis mean that DMD patients should always be considered at high surgical/anaesthetic risk and therefore management should be multi-

disciplinary and performed in a centre with the appropriate expertise. Surgical management of fractures should only be conducted at DMD-experienced centres which can provide the necessary anaesthetic and / or critical care support. Complex surgical procedures should only be done in hospital with critical care facilities.

A3. Guideline Statements

A3.1 Service Specification

Elective orthopaedic management should be conducted in conjunction with the patient's existing neuromuscular team which is likely to be at one of the North Star Centres (Appendix 1). It is also important to include their specialist physiotherapy and orthotics teams.

All long bone or disabling fractures in children and young people (< 19 years old) should be discussed for advice with a children's orthopaedic surgeon at a local designated children's trauma centre within 24 hours of presentation **(a list of children's trauma centres with necessary expertise and facilities is given in Appendix 2).** The patient's usual neuromuscular care team should be informed at the same time.

The management of fractures in adult patients (generally those who are 19 years and older) follows the same principles as in younger people and carries the same risks. We advise discussion with the regional specialist **children's** orthopaedic team wherever possible in view of their greater experience of the condition and ask them to liaise with the admitting adult service as appropriate. In all cases, fractures in DMD should be discussed with an orthopaedic specialist with expertise in managing DMD and/or neuromuscular disorders. The neuromuscular care team must be informed at the same time.

A3.2 Anaesthetic Considerations

A3.2.1 Perioperative steroid use – Most DMD patients take regular corticosteroids and are therefore at risk of adrenal insufficiency. For these patients, corticosteroid replacement is a vital part of perioperative management and local / national guidelines should be followed appropriately.

Detailed national guidance for management of paediatric adrenal insufficiency (including during the surgical procedures) has been developed by the British Society of Paediatric Endocrinology and Diabetes (Paediatric Adrenal Insufficiency Group On Behalf of the British Society of Paediatric Endocrinology & Diabetes, 2024).

Detailed national guidance for management of adult adrenal insufficiency during surgical procedures has been developed by the Society for Endocrinology, Associations of Anaesthetists and the Royal College of Physicians (Woodcock et al., 2020).

A3.2.2 Cardiopulmonary assessment – DMD results in progressive weakness of respiratory muscles and cardiac dysfunction. The most recent pulmonary and cardiac investigations should be reviewed (Bourke et al., 2022; Childs et al., 2023). Consider requesting up to date

investigations where appropriate as this will guide perioperative care including the need for critical care referral.

A3.2.3 Fat Embolus Syndrome (FES) management – Patients with DMD are at an increased risk of FES (Feder et al., 2017). Treatment of FES centres around supportive care by maintaining oxygenation and early fluid resuscitation. Management may include the requirement for mechanical ventilation and in such cases involve critical care early.

A3.2.4 Anaesthetic technique – Perioperative decision making should involve a senior anaesthetist with experience in managing DMD patients. Total Intra Venous Anaesthesia (TIVA) is the recommended anaesthetic technique as the use of succinylcholine and volatile anaesthetics are associated with a risk of severe hyperkalaemia and rhabdomyolysis.

The use of suxamethonium is contraindicated in DMD, as there is a risk of hyperkalaemic cardiac arrest.

Volatile agents should be avoided if possible, as there is a risk of rhabdomyolysis, and anaesthetic machines should be flushed prior to use. In exceptional circumstances volatile anaesthetics can be used, but the duration should be kept to a minimum and the anaesthetist should be prepared to manage life-threatening sequelae.

Regional techniques including spinal and epidural anaesthesia can be considered, but caution is needed where cardiac dysfunction / cardiomyopathy is present due to the effects of sympathetic blockade.

A3.3 Fracture Management (see also Part B)

Many fractures in DMD patients are minimally displaced and mechanically stable. In these cases, conservative management with immediate mobilisation with lightweight support using a cast or brace is indicated.

Adequate pain relief must be given throughout, initiated in the Emergency Department.

The use of multimodal analgesia is recommended to reduce the potential respiratory depressant effects of opiates in patients with reduced reserves. Use of NSAID should be for limited time periods only and gastroprotection should be provided. This is particularly important when combined with corticosteroids. Be aware that constipation is an increased risk in people with DMD and this can be exacerbated by opioid drugs (Marini Bettolo et al., 2015). Their use should be carefully considered.

Regional anaesthetic techniques may be indicated whether surgery is performed or not. Single nerve blocks or nerve infusion catheters should be considered. Ultrasound guidance is recommended where body habitus and/or joint contractures prove a challenge.

Ambulant patients with unstable long bone fractures **should be offered surgical stabilisation**. This is to enable immediate weight bearing or upper limb use to optimise muscle function, joint position, and maximise the chances of preserving independent

mobility and function. A discussion about potential risks and benefits with the patient/family is required.

In non-ambulant patients with an unstable long bone fracture, surgical stabilisation should also be considered and discussed with patients/families if causing problems with independence, positioning or pain management.

Once transfer to a specialist centre for surgical fracture management is agreed, the transfer should occur within 24 hours. The initial admitting centre should be provided with **written guidelines from the specialist centre** on immediate management prior to and covering transfer to the specialist centre.

Surgical stabilisation of long bone fractures should protect the full length of the bone. Combined intramedullary and plate fixation, or double plate fixation should be considered in metaphyseal fractures where bone fragility compromises stable intramedullary fixation.

Where casting is necessary, the knee should be cast in maximal extension and the ankle at 90 degrees (or maximum dorsiflexion if already restricted) to avoid flexion contractures. Casting should be done and applied in theatre or very early post-operatively.

Occupational therapy, physiotherapy and wheelchair services should be involved pre-operatively where possible to ensure appropriate seating and equipment for transfers are available post-operatively. Moving and handling assessments should be carried out as required.

Ensure early additional physiotherapy input to prevent contractures to injured and non-injured limbs during the recovery period. Intensive onward rehabilitation must be in place to regain independence. **A documented rehabilitation plan must be in the inpatient notes, shared with the patient / carers and passed on to community services.**

A3.4 Elective Surgery (see also Part C)

Elective surgery for contractures in DMD should only be conducted at specified centres which can provide the necessary anaesthetic and / or critical care support**.**

A4. Concluding remarks

This consensus guideline is important to ensure that safe, informed decisions are made by clinical teams, together with patients and families living with DMD in the event of long-bone fractures or decisions about elective surgery for contractures. Where available, published evidence has been used to support recommendations. Where this is not yet possible, expert experience and opinion of the multi-disciplinary working group (including patient representatives) provides helpful information on best practice. Endorsement from the BSCOS offers further validity. Implementation of these recommendations into clinical practice will harmonise and improve care for all people living with DMD, no matter in which centre they are seen. Next steps for this working group will include collaboration to collect data – for example on long bone fracture outcomes after casting vs internal fixation – and increase the evidence base to inform and support future updates to these guidelines.

Part B: DMD Long Bone Fracture Management

This document should be read in conjunction with PART A: Orthopaedic Guidelines on Management of Duchenne Muscular Dystrophy

B1. Introduction

Patients with Duchenne muscular dystrophy (DMD) are at increased risk of fracture due to bone fragility and may present with a long-bone fracture to any hospital with an Emergency Department (ED) (Birnkrant, Bushby, Bann, Alman, et al., 2018; Joseph et al., 2019; Phung et al., 2024; Quinlivan et al., 2010; Wong et al., 2023). While the technical aspects of fracture care in these patients require / are addressed by standard orthopaedic trauma techniques, particulars of their underlying condition, medical vulnerability and anaesthetic considerations mean that input from specialist centres is strongly advised. As stated in the Guideline (Part A), the advice is that all DMD patients with a long bone fracture should be discussed with a children's orthopaedic trauma specialist, and with the local neuromuscular team.

B2. Medical Background

Muscle function in patients with DMD deteriorates rapidly with immobility, and muscle contractures occur quickly if the muscles are left in a contracted position. Following a fracture, patients are therefore at risk of further-losing independent mobility and of developing knee and ankle joint contractures. Operative stabilisation with immediate weight bearing is consequently the preferred management of unstable fractures. In some circumstances fractures may be managed in a functional cast with immediate weight bearing but prolonged immobility is to be avoided and mobilisation without weight bearing is not practical.

DMD patients should be assumed to be on high dose corticosteroid treatment and therefore at risk of adrenal crisis (Wong et al., 2023). DMD patients are also at increased risk of fat embolus syndrome (FES) and should therefore have careful fluid balance monitoring, oxygen supplementation and be closely monitored for worsening hypoxia. Excessive oxygen administration in isolation should be avoided as it can worsen hypercapnia (Childs et al., 2023). There is some evidence that the risk of FES may be further increased by using a tourniquet and so the advice is to avoid tourniquet use where practical (Ida et al., 2018).

There are specific anaesthetic considerations which are dealt with in the main guidelines (Part A).

B3. Orthopaedic Fracture Management

Non-operative management is appropriate in the case of minimally displaced and mechanically stable fractures that can be satisfactorily treated in a lightweight cast or brace followed by immediate weight bearing. The joints **must not** be immobilised in a non-functional position, for example in an above knee cast with the knee flexed, or in a below knee cast with the ankle in equinus (as may be indicated in other patients with an apex posterior distal tibia fracture).

There should be a low threshold for operative stabilisation of unstable long bone fractures in patients with DMD.

Operative stabilisation should aim to protect the full length of a weight-bearing bone and be sufficiently robust to allow immediate function including weight bearing as tolerated. Upper limb fractures appear less common in UK studies of this patient group, presumably due to relatively low participation in activities that may lead to falls. Humerus shaft fractures may be managed in a brace in most instances, but if a patient is dependent on upper limb stability for wheelchair use, internal fixation with intramedullary nails is indicated, supplemented by plate fixation where necessary. Unstable forearm fractures are best managed with fixation at any age, elastic intramedullary nails are the most appropriate technique in growing patients, plate fixation with long plates would be standard treatment in skeletally mature. Mobilise the limb immediately post-operatively or following <5 days' protection in a cast or brace.

Lower limb fractures are the more common injuries in the DMD population, with distal femur fractures being the most frequent location. The antegrade elastic intramedullary fixation technique can be effective in younger patients with this injury (under 50 – 60 kg weight). If there are concerns about the stability of fixation due to position, configuration or bone fragility then supplementary plate fixation is indicated. It may be necessary to fix across the growth plate and in this case, management of subsequent growth disturbance should be anticipated. Mid-shaft fractures will be amenable to standard intramedullary fixation techniques depending on the size, weight and age of the patient. 'Growing' intramedullary devices such as the Fassier Duval system may be appropriate in younger patients but will require supplementary plate fixation or functional bracing to confer rotational stability and allow immediate function.

Femoral neck and proximal femur fractures occur particularly in non-ambulant patients who fall while transferring or as a result of wheelchair accidents. Operative treatment can be a challenge due to body habitus and bone fragility in this group, but stable anatomical reduction and fixation is still the goal to avoid the risk of avascular necrosis and allow the patient to sit up, transfer and be mobile in their chair. If a proximal femoral screw – plate is used, protect the rest of the femur with an intramedullary device in addition.
The use of post-operative casts or braces should be kept to a minimum to enable early functional rehabilitation. If joints have to be immobilised at all, keep the knee in extension in ambulant patients, and the ankle in neutral in all patients. Be aware that this patient group appear at increased risk of developing pressure sores within casts.

Part C: Elective Orthopaedic Management in Duchenne Muscular Dystrophy

This document should be read in conjunction with PART A: Orthopaedic Guidelines on Management of Duchenne Muscular Dystrophy

C1. The aim of elective orthopaedic surgical intervention in ambulatory patients is to maintain motor function for as long as possible. The aim of surgery in non-ambulatory patients is to allow comfortable, balanced sitting and functional upper limb use.

C2. Patients should have a home stretching programme and regular physiotherapy input to try to prevent joint contractures. Ankle-Foot Orthoses (AFOs) can be considered when there is reduced ankle dorsiflexion (consider night-time use in ambulatory and daytime use in non-ambulatory patients).

C3. A decision to proceed with surgery should involve the patient, family and multi-disciplinary team (physiotherapist, occupational therapist, orthotist, neurologist, community paediatrician, cardiologist, respiratory specialist, anaesthetist and orthopaedic surgeon).

C4. The patient should have a full respiratory and cardiac review before surgery (Bourke et al., 2022; Childs et al., 2023). A critical care bed should be available following surgery.

C5. In an ambulatory patient with equinovarus ankle contractures (together with good quadriceps and hip extensor strength), Achilles tendon lengthening can be considered. Regular post-operative physiotherapy input is needed. Surgery at the hip and knee level is generally not recommended.

C6. In patients in the early non-ambulatory phase with equinovarus ankle contractures, Achilles tendon lengthening may be considered to allow the feet to sit comfortably on wheelchair footplates or if unable to find comfortable footwear.

C7. Regular skin checks (with plaster removal) are required in the post-operative period to prevent pressure ulcers. Wound healing can be affected by long term treatment with oral corticosteroids and should be carefully monitored. Following plaster removal, a post-operative AFO may help prevent contracture recurrence.

C8. In the late non-ambulatory phase the risks of surgery are higher. However, surgery should still be considered after careful discussion particularly if the patient has significant pain or recurrent skin problems.

C9. The patient and family must be aware of the significant risks associated with surgery. These include wound infection and wound breakdown, worsening cardiac function, respiratory problems, pressure ulcers from plasters, pain, fat embolism and a small risk of death. The patient may need admission to a critical care facility following surgery and may require ventilatory support for an unknown length of time.

Disclaimer: The guidance herein is expert opinion drawn from national multidisciplinary experience and published research, but the working group acknowledges that there are no randomised controlled trials to support it.

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Orthopaedic: O; Neuromuscular: NM; Patient rep: PR; Endocrine: E; Physio: PT; Orthotics, OR; Anaesthetics: A

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Appendix 1: List of Specialist Paediatric Neuromuscular Centres in the North Star Network

Aberdeen Royal Infirmary
Belfast: Royal Belfast Hospital for Sick Children
Birmingham Children's Hospital
Bristol Children's Hospital
Cambridge: Addenbrooke's Hospital
Cardiff: University Hospital of Wales
Dundee Ninewells Hospital
Edinburgh: Royal Hospital for Children and Young People
Glasgow: Royal Hospital for Children
Leeds General Infirmary
Leicester Royal Infirmary
Liverpool: Alder Hey Children's Hospital
London: Evelina Children's Hospital
London: Great Ormond Street Hospital
Manchester Children's Hospital
Newcastle-upon-Tyne: Royal Victoria Infirmary
Nottingham: Queens Medial Centre
Oxford: John Radcliffe Hospital
Preston: Royal Preston Hospital
Sheffield Children's Hospital
Southampton Children's Hospital
Swansea: Morriston Hospital

Appendix 2: List of hospitals with specialist children's trauma care suitable for management of fractures in Duchenne muscular dystrophy patients.

Aberdeen Royal Infirmary
Addenbrooke's Hospital, Cambridge
Alder Hey Children's Hospital, Liverpool
Birmingham Children's Hospital
Bristol Children's Hospital
University Hospitals, Plymouth
Dundee Ninewells Hospital
Evelina London Children's Hospital
Great Ormond Street Hospital
John Radcliffe Hospital, Oxford
Leeds General Infirmary
Leicester Royal Infirmary
Manchester Children's Hospital
Norfolk and Norwich Hospital
Queens Medial Centre, Nottingham
Royal Belfast Hospital for Sick Children
Royal Hospital for Children and Young People, Edinburgh
Royal Hospital for Children in Glasgow
Royal Stoke Hospital
Royal Sussex County Hospital
Royal Victoria Infirmary, Newcastle upon Tyne
Sheffield Children's Hospital
Southampton Children's Hospital
University Hospital of Wales, Cardiff