CORTICOSTEROIDS IN DUCHENNE MUSCULAR DYSTROPHY:

DMD Care UK standard of care guideline

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Some clarifications on terminology and scope

These guidelines refer to the use of corticosteroids (deflazacort, prednisolone and vamorolone) in people with Duchenne muscular dystrophy (DMD). While some of the information might also apply to rare females with DMD treated with corticosteroids, the lack of evidence in this population makes the development of any specific guidance very difficult.

Throughout this document, the term corticosteroids (CS) **refers to all corticosteroids, including vamorolone**, unless otherwise specified.

Until recently, the CS used in the UK for treatment of DMD were deflazacort and prednisolone - referred to here as 'classic CS'.

Prednisolone is the formulation available in the UK. It is very similar to prednisone used in some other countries including the US. Prednisone is converted by liver enzymes to prednisolone before it takes effect.

Where we refer to use of vamorolone in patients with DMD, this is always daily use.

A: Benefits of corticosteroids in DMD

CS treatment (traditionally prednisolone or deflazacort) is recommended as the established standard of care for DMD (1,2).

The first clinical trial on the efficacy of CS in DMD was published in 1989 (3); several subsequent studies have confirmed their capacity to defer the progression of the disease throughout an individual's lifespan (1,4–11).

The benefit of CS on muscle strength and function in DMD varies from person to person and depends on several factors, including the age when CS treatment is initiated, the baseline muscle function, the CS regimen etc.

A summary of the acknowledged benefits of classic CS in DMD is given in Box 1.

Addition of vamorolone as a CS treatment option

Vamorolone is a more recent steroid designed to treat DMD and potentially other inflammatory conditions. Vamorolone was approved by NICE as an option for treating people with DMD aged 4 years and over in January 2025 and it now adds a third option when considering CS treatment for DMD as part of the standard of care (www.nice.org.uk/guidance/ta1031).

Vamorolone is described as a "dissociative corticosteroid" as it is intended to retain the therapeutic benefits of classic corticosteroids, whilst reducing some of the side effects (12). A summary of the benefits of vamorolone is given in Box 2.

Vamorolone has similar anti-inflammatory action to prednisolone and deflazacort (*transrepression* activity as it is mediated by *repression* of a specific inflammatory pathway, NF-κB).

Vamorolone lacks the affinity for the glucocorticoid response element (GRE) binding site, which is thought to be responsible for some CS side effects.

In contrast to classic CS, vamorolone is an antagonist of the mineralocorticoid receptor (MRA), like spironolactone. As such it should NOT be used for sick day dosing (see Table 1). The potential cardioprotective benefits of vamorolone still need to be investigated.

Classic CS have been used for treating DMD since the late 1990s. Therefore, there are extensive data on their short- and long-term effect on muscle strength and function in DMD. In contrast, only data on short / medium term exposure to vamorolone in ambulant children with DMD have been published to date (6 months in a prednisone placebo-controlled study and up 30 months in an open label study of prednisone versus matched external control group) (13–15).

Longer term safety and efficacy of vamorolone is not yet available. Moreover, evidence so far is limited to vamorolone use in young, steroid naïve, boys with DMD. There is no data on the effect (safety and efficacy) of switching to vamorolone after receiving long term prednisolone or deflazacort. Neither is there data on its use in older, non-ambulant patients.

Further evidence on the long-term efficacy and safety profile of vamorolone in DMD, in those of different ages and at different stages of the condition, is expected to emerge in the coming years, following the approval of vamorolone use in a broad population of patients with DMD in several countries.

Box 1: Benefits of classic corticosteroids in DMD

Classic CS (prednisolone or deflazacort), administered daily or intermittently in DMD, have been shown to:

- **Improve muscle strength and motor function in lower limbs**, as measured by time to rise from the floor, time to walk/run 10 meters and the North Star Ambulatory Assessment (NSAA) (7,16). When initiated early (before the age of 6 years) and using daily regimens, CS can lead to an improvement in muscle strength, ability to acquire new motor skills (e.g. jumping), followed by a delayed decline compared to steroid naïve patients. The improvement can be noted as early as within 3 months of treatment initiation and can continue for up to 18 months after initiation of treatment (9,17).
- **Delay loss of ambulation** by approximately 3 years (7).
- **Delay loss of upper limb function**, as measured by the ability to reach overhead, to reach hands to mouth and to be able to functionally use the hands (18).
- **Decelerate the decline of respiratory function** (5,6,19,20).
- **Delay the onset and slow the progression of cardiomyopathy** (6,21).
- **Delay or prevent the development of severe scoliosis** requiring surgical intervention (10,11).
- **Prolong survival**. The implementation of multi-disciplinary standards of care including CS treatment, timed respiratory support and pro-active cardiac interventions have significantly improved life expectancy to the current median of 28 years (22).

BOX 2: Benefits of vamorolone in DMD

- Daily vamorolone has shown similar efficacy to daily prednisone across various functional outcome measures at 6 months* (15). Vamorolone has also been shown to have similar efficacy when compared to historical control cohorts treated with different CS types and regimens up to 30 months** (13).
- Vamorolone at 2 mg/kg/day is less effective than vamorolone at 6 mg/kg/day, with the lower dose resulting in less improvement on the majority of the motor function outcome measures at 12 months of treatment (14).
- Longer term efficacy comparison is not yet available.
- There is no published efficacy comparison between vamorolone and deflazacort or with intermittent CS regimens.
- Improvements seen after 6 months of daily prednisone treatment in steroid naïve patients were sustained when 'switching' to vamorolone 6 mg/kg/day (14). There is no published information available on efficacy of switching to vamorolone after receiving longer term prednisone or deflazacort (14).
- Whilst there is currently no evidence on the efficacy of vamorolone on cardiac and respiratory outcomes in DMD, there is no reason to expect that it will be significantly different to that seen with deflazacort or prednisolone. This should also be the case for the impact of vamorolone on other complications of DMD such as scoliosis.

***** VISION DMD Study, *vamorolone 6 mg/kg/day versus prednisone 0.75 mg/kg/day at 6 months after treatment initiation*; outcome measures: time to rise from the floor, 6-minute walking distance, time to run/walk 10 meters, time to climb 4 steps, NSAA total score

** LTE study, vamorolone vs different CS (any type, dose and regimes) compared to CINRG natural history and UK North Star cohort. Outcome measures: time to rise from the floor, 6-minute walking distance, time to run/walk 10 meters, time to climb 4 steps, NSAA total score

B: Considerations for side effects of corticosteroids in DMD

1. All CS types and regimens are associated with side effects.

2. The most common of these are reported in Box 3.

3. Clinical experience indicates that side effects vary significantly between individuals, both in their severity and time of onset after treatment initiation. These variabilities should be made clear to families.

4. Not all patients will experience all side effects, but the risk increases with cumulative doses. Risk and severity of side effects therefore increases with dose, duration of treatment, regimen, but also susceptibility factors which are specific to the individual.

5. The significance and burden of the same side effect can vary between individuals, between families and over time in the same individual. This reflects different demographics, psychosocial factors, growth, physical decline over time and personal priorities. For example, the impact on appearance (Cushingoid features, weight gain, stunting of growth, delayed puberty) may be more important for patients during adolescence, and height may become less important after loss of ambulation.

6. The impact of CS on bone health and the increased risk of fractures is often a major concern for parents and can significantly impact ambulatory status.

7. Some side effects (e.g. weight gain, bone health, delayed puberty) can be mitigated by specific interventions whilst others may be more difficult to manage and have no reliable interventions (e.g. stunting of growth). See Table 1.

BOX 3: Side effect profile of corticosteroids in DMD

CS side effects that may be observed within the first 6 months from treatment initiation:

- Adrenal insufficiency
- Behavioural changes (e.g. temper-tantrums; emotional behaviour; aggressiveness; insomnia)
- Weight gain
- Gastroesophageal reflux and epigastric pain
- Immunosuppression (increased risk of infections)
- Hypertension

Additional side effects more related to cumulative dose exposure*:

- Cushingoid appearance
- Stunting of growth**
- Bone demineralization and increased fracture risk
- Delayed puberty
- Other gastro-intestinal symptoms (e.g. peptic ulcer disease)
- Cataracts
- Excessive hair growth (hypertrichosis)
- Skin infections
- Other skin changes (e.g. skin fragility)
- Hyperglycemia

*Only data up to 30 months of exposure to vamorolone has been published to date; thus the impact of vamorolone on many side effects related to cumulative dose exposure (e.g. delayed puberty, cataracts, insulin resistance, skin changes) is not yet known
** Stunting of growth has not been observed in boys treated with vamorolone up to 30 months after treatment initiation, whilst it is well described with prednisolone and deflazacort.

C: Corticosteroids in DMD: General principles for clinical care

1. CS should be discussed with all families diagnosed with DMD. This should usually be at the time of (or soon after) diagnosis. While it is recognised that CS are associated with several side effects, the effect on motor function, respiratory and cardiac function and overall survival is well established and justifies the routine recommendation for treatment (refer to Boxes 1-2).

2. The decision about CS is a process. The impact of a recent diagnosis of DMD on the ability of families to process information should be taken into account. Information should be clear and may need to be given in stages so as not to overwhelm the family. Supplementing the discussion with clear written information is recommended to facilitate decision-making and ensure family trust and engagement (a template letter for families is given in Appendix C and for GPs in Appendix D). Families should be signposted to valid and reliable sources of information, including the DMD Care UK corticosteroid guideline for families (www.dmdcareuk.org).

3. Decisions about CS (initiation/regimen/type) can be difficult because it is often seen as a choice or 'trade-off' between different adverse consequences (weighing up loss of function against significant side effects or uncertainties).

4. Family and patient priorities will vary and should be explored and discussed. The balance of benefits versus side effects will not be the same for all patients and families or at different stages of life.

5. The same CS may not be right for everyone and indeed the optimum choice of CS might change over time and based on individual response and side effects.

6. We aim to prioritise some CS regimens for clinical practice and harmonise care based on current evidence. It is recognised, however, that there is a lack of robust evidence (e.g. from randomized, controlled studies) on long term outcomes (both benefits and side effects) of different CS types and regimens. This means that there are limitations in consulting/advising families. This should be made clear during discussions.

7. It is important that this guidance is reviewed regularly and updated as new data, particularly on vamorolone, becomes available and that these data are clearly explained to patients.

8. Emerging data in non-ambulatory and adult patients supports continuation of CS treatment after loss of ambulation to delay loss of function in the upper limbs and to slow decline in respiratory and cardiac function. Evidence regarding optimal CS type/regimen and dose in these populations is lacking however and may depend on their side effect profile.

9. Families should be educated about adrenal suppression as soon as any CS, are prescribed, and understanding must be checked/refreshed in clinic. Provision of home-dose emergency hydrocortisone must be discussed and offered as per the standards of care (23). Once CS treatment is initiated, families should be encouraged to use the Emergency Care app, steroid alert cards and alert wristbands (www.dmdcareuk.org).

10. CS must not be discontinued suddenly or withdrawn during an acute illness (e.g. chest infection) due to the risk of adrenal crisis and possible impact on respiratory function. Extra CS might be needed during acute illness (23) . The decision to withdraw treatment should be discussed with the neuromuscular specialist with the involvement of a multi-disciplinary team, including the endocrine team, to advise on the process for withdrawing.

11. Key considerations for switching from classic CS to vamorolone are provided in the UK national guidance on "clinical monitoring of bone and endocrine outcomes in individuals with DMD on vamorolone" developed by the Endocrine & Bone Working Group of DMD Care UK (24).

D: Treatment initiation

1. Based on current knowledge, CS should be initiated between the age of 4-6 years and early treatment initiation (4-5 years) is recommended to optimise benefit. There is insufficient evidence at the current time to justify treatment initiation before the age of 4 years, in view of the potential long-term side effect profile.

2. Initiation of treatment < 4 years may be considered in individual cases, including where it may be a factor for participation in clinical trials. Note that vamorolone is only approved in the UK for people aged 4 and older.

3. CS may sometimes be initiated later than 6 years of age (e.g. because of late diagnosis or parental choice). Initiation of treatment after the age of 6 years is still recommended but may not be as beneficial (e.g. it might not result in an improvement of motor performances but rather in stabilization or delayed decline of muscle function). This should be discussed with families to manage expectations.

4. Initiation of treatment in non-ambulatory patients might be considered in individual cases to slow down decline in upper limb and/or respiratory function although there are only limited data to reliably inform the expected benefit and side effect profile in this population.

5. CS should usually be taken in the morning to mimic the circadian rhythm (although evidence for this is sparse and it should be discussed on an individual basis). They should always be taken on a full stomach to reduce the risk of gastro-intestinal disturbance. Evening dosing may sometimes be considered in case of behavioural issues, but clinicians should discuss the possibility of this causing insomnia.

6. Family compliance and ability to manage adverse events need to be considered and treatment might need to be delayed until measures are in place to support the family. Lack of compliance with treatment can represent a contraindication to all CS because of the associated risk of adrenal crisis if treatment is not taken as prescribed.

7. Presence of pre-existing behavioural problems or learning difficulties are not an absolute contraindication to CS. Families should be informed about the possibility of a deterioration in behaviour at the time of initiation of CS and access to additional psychosocial support should be considered.

E: Corticosteroid type and regimen

1. At treatment initiation (between 4-6 years), the recommended regimen is daily (prednisolone, deflazacort or vamorolone). This recommendation is based on current knowledge, showing that daily regimens of both prednisolone and deflazacort are associated with better effects on motor function than prednisolone 10 days on/10 days off. For daily use, there is no significant difference in benefits between prednisolone and deflazacort over the first 3 years of treatment. Vamorolone has only be tested as a daily treatment and it is therefore recommended that it should only be prescribed on this basis. Daily vamorolone at a dose of 6 mg/kg/day has shown similar efficacy to classic CS across various functional outcome measures at 6 months and up to 30 months (see Box 2) (13–15). Box 5 summarises baseline checks recommended prior initiation of CS treatment in DMD.

2. Some observational studies that included older patients suggest better motor function in patients treated with deflazacort compared to prednisolone, however these findings are not confirmed in other studies. Prednisolone (or deflazacort) 10 days on/10 days off has been used in clinical practice and might be considered as an alternative, based on family preference and following a careful discussion about the evidence on differences in benefits and side effects profile (refer to Box 1 and Table 1).

3. Currently there is not enough evidence to support the use of other regimens. The lack of robust, long-term, longitudinal data on their use means it is difficult to advise families about expected benefits and side effects and makes management more difficult and arbitrary. Intermittent regimes of vamorolone have not been tested and are not recommended in clinical practice.

3. Patients who are not on a daily CS regimen, should be reviewed and consideration given to switching them to daily CS (see Box 4). This should be on a case-by-case basis, taking into account expected benefits, functional status, side effect profile and family/patient preferences. The potential impact of alternative regimens on clinical trial eligibility should form part of the discussion with families.

4. Side effect profiles differ between CS types and regimens, but there are limited data to compare their long-term side effect profiles (Table 1). The long-term side effect profile of vamorolone compared to prednisone and deflazacort is unknown. Moreover, there is insufficient evidence about the impact of changing CS type (including to/from vamorolone) and CS regimen on efficacy and side effects after long term CS treatment which makes consultation with patients and families difficult.

5. The side effects of CS and the comparison across different types and regimens are summarised in Table 1. Vamorolone has not been associated with growth failure and has been shown to have less negative impact on bone health compared to daily prednisolone and deflazacort and might therefore be preferred in young children. Deflazacort is associated with less weight gain compared to prednisolone and might be preferred for children with pre-existing high BMI at the time of treatment initiation. Switching from one CS to another (e.g. prednisolone to deflazacort; prednisolone or deflazacort to vamorolone) and vice versa may be justified in an effort to manage side effects, e.g. in patients for whom

weight gain is the major side effect or concern. However, the patient and the family should be made aware of the potential impact on other side effects (see Table 1).

6. Switching from daily to 10 days on/10 days off regimen is not advisable due to the risk of adrenal crisis and steroid withdrawal syndrome (Box 4). It might be considered in individual cases to manage side effects but should always be discussed with an endocrinologist. The patient and the family should be made aware of the potential impact on functional outcomes and the lack of evidence about differences in some side effects (see Table 1).

7. The risk of adrenal withdrawal syndrome is dose dependent and should also be considered when switching from prednisolone or deflazacort to vamorolone. Refer to "clinical monitoring of bone and endocrine outcomes in individuals with DMD on vamorolone" for guidance on switching (24).

8. At the time of switching from and to any CS type and/or regimen, perform the 6-monthly assessments (see Box 6) to collect baseline measures. If switching to vamorolone (see also Appendix A), ensure to have up to date blood tests for liver function and electrolytes in individuals on mineralocorticoid receptor antagonists (e.g. eplerenone, spironolactone).

BOX 4 – Considerations when switching corticosteroid type or regimen

- There are no concerns in switching from prednisolone to deflazacort or vice versa and the change can be done from one day to another without need of tapering. The dose might however be adjusted (0.75 mg of prednisolone: 0.9 mg of deflazacort).
- The equivalent dosing of prednisolone or deflazacort with vamorolone is unknown. Risk of adrenal withdrawal syndrome must be considered when switching from prednisolone or deflazacort to vamorolone, and it is dose dependent.
- There are no concerns in switching from 10 days on/10 days off CS to daily regimens and the change does not require tapering.
- Switching from daily to 10 days on/10 days off regimen is not advised and is more problematic due to the risk of adrenal crisis and steroid withdrawal syndrome and will require careful tapering. This must be discussed in advance with an endocrinologist.
- When switching from one CS type to another or from one regimen to another, the impact of benefits and side effects should always be discussed with the patient/family.
- Based on clinical experience and expert opinion, in the short term, the impact of side effects is likely to be limited to weight (more weight gain with prednisolone compared to deflazacort) and behaviour (possible worse effect with prednisolone than deflazacort) while the differences in other side effects may only become apparent after long term exposure (months/years). However, there is a lack of evidence on the effects on benefits and side effects from switching CS type or regimen.
- Based on current evidence, switching from prednisolone to vamorolone after a short treatment period, is likely to result in an improvement of linear growth (in children) and in bone health. However, the effect of switching from prednisolone or deflazacort to vamorolone after long-term exposure is unknown.
- The potential impact on benefit and side effects of switching to the recommended dose of vamorolone (6 mg/kg/day or 240 mg/day if body weight > 40 kg), should be discussed with the family (see Appendix C).

F: Dose

1. When initiated, CS should be prescribed at their optimal dose (0.75 mg/kg/day of prednisolone or 0.9 mg/kg/day of deflazacort or 6 mg/kg/day of vamorolone) to allow maximum benefit and early identification of non-responders. If started on a lower dose and no clear clinical benefit is seen, it will be difficult to judge whether this is due to the inappropriate dose or to lack of treatment response (see Section J).

2. Dose should be reviewed at each clinical assessment, based on recent weight. In ambulant boys, doses should be maintained as close as possible to the recommended

dose/kg (but not exceeding the maximum recommended dose – see point 4 below) based on the balance of benefits and side effects (see Table 1).

3. There is some limited evidence that in ambulant patients, doses below 0.3 mg/kg/day of prednisolone (0.4 mg/kg/day of deflazacort) are sub-therapeutic as they are associated with less effect on muscle strength and function but can still cause side effects. Similarly, in ambulant patients, vamorolone 2 mg/kg/day is less effective than daily prednisolone and vamorolone 6 mg/kg/day.

4. Maximum recommended doses are prednisolone 30 mg/day, deflazacort 36 mg/day (1) and vamorolone 240 mg/day (www.nice.org.uk/guidance/ta1031).

5. Currently there is no guidance on the optimal dose of CS in non-ambulatory patients and adults with DMD (See section H).

6. Currently there is no international consensus on CS-dose adjustments in response to side effects. In the absence of robust evidence-based recommendations, the published guidance from the FOR DMD study can be used as a reference as they have shown overall good CS tolerability over at least the first three years of treatment (16,25).

7. There is limited evidence regarding the impact of dose reduction or discontinuation on side effects, after long term CS treatment, which makes consultation with patients and families difficult. Clinical experience suggests that some side effects (e.g. weight gain, Cushingoid appearance, behavioural issues triggered by CS) may improve after dose reduction or discontinuation. However, they are rarely completely reversible, and the degree of any improvement varies from patient to patient, time of exposure to CS, age and other individual risk factors, and cannot therefore be predicted. This needs to be explained clearly to families. Further studies/data are required to provide evidence to support these clinical observations.

G: Monitoring and management

1. Patients on CS should be monitored for treatment response and development and management of side effects with clinical and functional assessments performed at least 6-monthly. More frequent neuromuscular assessments are recommended at the time of initiation of CS (e.g. baseline, 3 and 6 months) to allow evaluation of benefits and early onset side effects and to implement established, prompt interventions if required (refer to Box 5 and 6). CS dose and regimen adjustments in response to these evaluations, and consequently clinical monitoring, should be tailored to individual patients depending on their specific circumstances. See also Appendix B (15).

BOX 5 – Good practice: baseline check list prior initiation of CS treatment in DMD

- Family and medical history (specifically focused on family history of diabetes, tuberculosis (TB), and medical history of chicken pox immunisation)
- Up to date national immunisation schedule, including pneumococcal 23 polyvalent (20).
- Establish varicella and measles immunity. If IgG antibodies are not detected, the patient will require immunisation according to the national guidance (https://www.gov.uk/government/publications/measles-the-green-book-chapter-21 and https://www.gov.uk/government/publications/varicella-the-green-book-chapter-34). CS can be initiated after completing full immunisation, even if IgG are not detected. However, specialist advice should be sought in case of future exposure in children who remain IgG negative. Assess for TB risk according to national guidance and if there are concerns, refer to a specialist for advice.
- Vital signs (including height, weight, heart rate, blood pressure).
- Medical history and physical examination (specifically focused on symptoms and signs of gastroesophageal reflux disease (GORD).
- Motor function assessment (e.g. NSAA, timed function test).
- Respiratory assessment (see respiratory guidelines (20)).
- Blood tests for haematology (including differential) and biochemistry (including electrolytes, urea, creatinine, liver function* (particularly for vamorolone to allow dose adjustment in case of liver impairment), bone profile, Vitamin D).
- Urine dipstick to check for presence of glucose
- Discuss with endocrinologist if family history of metabolic disorder (e.g. diabetes)
- Cardiac assessment (see cardiac guidelines (21)).
- Eye assessment (by an ophthalmologist/optician) to exclude pre-existing glaucoma or cataracts.
- Bone health monitoring including baseline lateral thoracolumbar spine imaging (either with spine radiographs or lateral DXA based assessment) to assess presence of pre-existing vertebral fractures (see endocrine guideline). Baseline spine DXA scan to evaluate bone density should be arranged for children aged 5 years and above.
- Vitamin D supplements should be prescribed routinely at the time of CS treatment initiation according to national recommendations.
- Dietary and behavioural advice should be given. Further information on DMD specific guidance is expected in the future from ongoing research in these areas (23,26).
- Provide a written emergency sick day plan to be followed during acute illness and educate the family about sick day plan and signs, symptoms and management of an adrenal crisis (23,26).

** Standard liver function might be difficult to interpret in view of the underlying muscle condition - consider monitoring with GGT, bilirubin, albumin and coagulation screening (PT, INR).*

BOX 6 – Good practice: check list for routine 6-monthly neuromuscular clinics

- Vital signs (including height, weight, heart rate, blood pressure). Accurate assessment of height can be difficult, especially in non-ambulatory patients (see Box 2 in (20) for guidance.
- Weight – must be checked at each follow-up appointment for safety and drug dose adjustments; a hoist or a wheelchair scale must be available in any clinical setting where patients with DMD are followed up; if appropriate/needed, patients should be reminded to bring their hoist sling in clinic at each follow up assessment.
- Height – must be checked at each follow up appointment. It is required for evaluation of respiratory function. Accurate assessment of height can be difficult, especially in non-ambulatory patients (see Box 2 in (20)) for guidance.
- Blood pressure (BP)– close monitoring with monthly measurements during the first three months from CS initiation to exclude acute hypertension followed by 6-monthly monitoring in clinic. Refer to the ESH guidelines for BP measurements (27).
- Medical history and physical examination (specifically focused on symptoms and signs of gastro-oesophogeal reflux disease - GORD).
- Motor function assessment (e.g. NSAA, timed function tests, Brooke scale and/or PUL 2.0)
- Respiratory assessment (see respiratory guidelines (20)).
- Annual blood tests for haematology (including differential) and biochemistry (including electrolytes, urea, creatinine, liver function*, bone profile, Vitamin D, magnesium and vitamin B12 if on omeprazole.
- Urine dipstick - close monitoring with monthly measurements during the first three months from CS initiation to monitor presence of glucose following by 6-monthly monitoring in clinic.
- Annual eye assessment (by an ophthalmologist/optician).
- Annual bone health monitoring (see endocrine guidelines (23).
- Dietary and behavioural advice should be reinforced if appropriate.
- Annual cardiac assessment (see cardiac guidelines (21)).
- Review and update the written emergency sick day plan and reiterate information about signs, symptoms and management of an adrenal crisis.

** Standard liver function might be difficult to interpret in view of the underlying muscle condition - consider monitoring with GGT, bilirubin, albumin and coagulation screening (PT, INR).*

H: Corticosteroids after loss of ambulation (LOA) and in adults

1. Emerging data in non-ambulatory and adult patients supports continuation of CS treatment after LOA to delay decline of upper limb, respiratory and cardiac function. Although there is no evidence yet on the effect of vamorolone after LOA, it is reasonable to

think that continuation of vamorolone after LOA will show similar effect on clinical outcomes as classic CS (upper limb, respiratory and cardiac function).

2. There are no data on the recommended CS dose and regimen after LOA and/or in adulthood. Further research is required on this.

3. Once patients reach LOA, they are usually on a lower dose than that recommended at treatment initiation. For classic CS, there is consensus that after LOA doses might be kept around one third lower than the recommended dose (that equals to approximately 0.5 mg/kg/day of prednisolone and 0.6 mg/kg/day deflazacort) but this is based on expert opinion only and should be evaluated case by case. There are no data to support the recommendation around a minimum effective dose in adults.

4. There are no data on the dose of vamorolone that might be appropriate for adults. However, if switching from prednisolone or deflazacort to vamorolone in adults, the recommended dose of 6 mg/kg/day or 240 mg/day in patients weighing 40 kg and above is recommended to reduce risk of adrenal suppression. The dose can be subsequently tapered (see endocrine guidelines (23,26)) The potential risk of side effects should be carefully discussed with the patient and their families.

5. In clinical practice, the dose is often maintained after LOA and in adulthood and it is not adjusted per kg of body weight thereafter. Dose must be evaluated at each clinical review. Dose adjustments should be driven by side effects that are not manageable and/or distressing to the patients and on benefits to muscle function, in particular upper limbs and respiratory function.

6. Initiation of treatment in non-ambulatory patients, previously CS naïve or reintroduction in those who discontinued CS, might be considered in individual cases to slow down decline in upper limb and/or respiratory function. In the absence of evidence, potential benefits need to be balanced and weighed against the different side effects. In older patients this can be difficult and should be discussed with a clinician with experience in treating adults with DMD. The advantage of one CS against the other in the adult population should be evaluated on a case-by-case basis.

I: Treatment discontinuation

1. The decision to discontinue CS should be in discussion between the clinical team and patient/family and should always be patient-guided based on assessment of benefits/side effects.

2. If a decision to discontinue CS is made:

- Discontinuation should never be sudden with any CS. Weaning, with testing of adrenal axis, is needed and must be discussed with an endocrinologist.
- Patients and families must be advised about the risk of adrenal suppression and should continue to be provided with emergency steroids according to the endocrine

guidance on management of adrenal suppression (23). Vamorolone causes adrenal suppression, similarly to prednisolone and deflazacort.

- Expectation about the impact of discontinuation on established side effects should be carefully managed and discussed with the patient and the family before starting tapering.

3. Patients and families should be informed about the possible deterioration of muscle strength and function, including respiratory function, after treatment discontinuation. Closer follow up (e.g. 3 months after initiation of tapering) should be considered to monitor changes in muscle and respiratory function and to guide management and interventions (e.g. a decision whether to maintain the patient on lower doses of CS; a referral to respiratory team if criteria are met).

J: A note on 'non-responders'

It is recognised that some boys do not seem to benefit from CS as expected based on the available natural history and clinical trial data. These patients (non-/poor- corticosteroid responders) are complex and their management and clinical care can be difficult. Due to the inter-individual variability of response to CS and associated factors, it is very difficult in clinical practice to discriminate whether a patient is not benefitting at all from CS; moreover, there are no data on whether the absent/poor response to CS on skeletal muscles accurately predicts the response on the respiratory and/or cardiac function. Clinical experience however shows that even the non-/poor- CS responders can still develop CS related side effects.

As CS treatment is always a balance between benefits and side effects, it is important that discontinuation of CS is considered in these cases and carefully discussed with patients and families. In the absence of other valid treatment options, it is recognised that the decision to discontinue CS in these cases can be very difficult and upsetting for families. If the decision is made to discontinue CS, closer monitoring of muscle and respiratory function is recommended to promptly identify changes during CS tapering/discontinuation which can further help in understanding the response to CS and support the family.

Table 1: CS side-effect considerations (see also Appendix B, (15) **for further guidance)**

Side effects	Comments	Comparison between types and regimens	Monitoring	Management and Intervention
Adrenal insufficiency	All people with DMD prescribed with CS, including vamorolone and regardless of regimen, should be assumed to have adrenal insufficiency and therefore to be at risk of adrenal crisis during acute illness, emergencies or significant stress.	The risk of adrenal insufficiency occurs with all CS types and regimens in a dose dependent manner.	Testing of the adrenal axis during treatment with CS (any type or regimen) is not routinely recommended. Testing of adrenal axis at the time of discontinuation of CS is mandated and must be discussed with an endocrinologist. Refer to *clinical monitoring of bone and endocrine outcomes in individuals with DMD on vamorolone* for guidance when switching from prednisolone/ deflazacort to vamorolone. (24) Swapping from daily CS to intermittent 10 days on/10 days off regimen should	Ensure a steroid sick day plan for moderate and severe acute illness is in place. Refer to the DMD Care UK recommendation of endocrine & bone monitoring in DMD (26). Vamorolone should NOT be used for sick day dosing; it may lead to electrolyte abnormalities, due to the fact that vamorolone is a mineralocorticoid receptor antagonist. Refer to *DMD Care UK - Clinical monitoring of bone and endocrine outcomes in individuals with DMD on vamorolone* for guidance on switching. (24)

			not be routinely considered. If this is deemed clinically appropriate, it must be discussed with an endocrinologist.	
Weight gain	Weight gain occurs commonly with all CS types and regimens. Time of onset and severity can significantly vary from patient to patient. It can be severe enough to require dose reductions and is one of the most common causes for treatment discontinuation.	Daily deflazacort is associated with less weight gain than prednisolone (either daily or 10 days on/10 days off), even though weight gain still occurs with deflazacort treatment. Vamorolone is associated with similar weight gain and increase in body mass index to daily prednisolone.	Close vigilance (particularly if patient, parents, or siblings are obese). Reinforce dietary advice before starting steroids and at each clinical assessment (to the entire family, not just the patient). Alert families about increased appetite at the time of starting CS.	Deflazacort might be considered as first choice if there is a pre-existing predisposition to obesity. Dose adjustments of CS might be considered for balancing muscle outcomes and wishes of the family (see Appendix B for guidance). Consider changing from prednisolone to deflazacort if severe weight gain on CS. The impact of changing from deflazacort to vamorolone on weight is unknown. Consider discussion with endocrinology if there is

				significant weight gain/obesity. Guidelines about weight management are currently in development by the DMD Care UK nutrition working group.
Growth failure and short stature	This is a very common side effect of prednisolone and deflazacort, and it can be severe and distressing for patients, especially during ambulatory stages. In contrast, boys on vamorolone treated for up to 30 months did not show decline in height percentiles.	Daily regimens of prednisolone and deflazacort cause greater degree of growth failure compared to prednisolone 10 days on/10 days off. Daily deflazacort is associated with a worse impact on growth. However, none of the classic CS regimens (prednisolone or deflazacort, daily or 10 days on/10 days off) can completely prevent growth failure In contrast, vamorolone is not associated with growth failure.	Monitor growth (height) at each follow up assessment. In non-ambulant patients, measurement of body segment (e.g. ulnar length or arm span) should be performed. However, estimation of height from measurement of body segment generally over-estimates height in CS-treated boys with DMD Box 2 of respiratory guidelines (20). Monitor for the development of scoliosis (clinical assessment; antero-posterior spine x-ray at the time of LOA or if	Vamorolone might be considered in young children to avoid growth failure and in pre-pubertal children, when growth failure is distressing for the child. If there are significant concerns regarding linear growth, consider referral to endocrinology for a growth assessment. In those aged 12 years or older, consider referral to endocrinology for testosterone therapy if appropriate as this may lead to some improvement in growth rate.

		The negative effect of prednisolone on growth seems to partially recover after switching to vamorolone, after short exposure (first 6 months of treatment). The effect of switching to vamorolone after longer exposure to classic CS on growth is unknown.	a curve is observed on examination or if clinical assessment is inadequate (e.g. severe obesity).	Growth hormone therapy is not recommended to improve height in DMD unless GH deficiency is diagnosed. Significant catch-up growth does not occur, likely due to growth hormone resistance. Clinically significant side effects (including type 2 diabetes and benign intracranial hypertension) have been reported.
Osteoporosis and fragility fractures	Long term use of CS increases fracture risk, including long bone and vertebral fractures. Vertebral fractures can be asymptomatic and have been reported as early as six months after starting treatment. The risk of fractures increase with dose and duration of CS exposure.	Daily regimens are associated with a higher risk of fractures compared with 10 days on/10 days off. Daily deflazacort is not bone sparing and is associated with a comparable fracture risk to daily prednisolone. In contrast to prednisolone and deflazacort, vamorolone does not suppress bone	Annual screen for vertebral fracture with lateral thoracic and lumbar spine radiographs or lateral DXA based vertebral fracture assessment. Prescribe Vitamin D supplement at the time of CS initiation, Annual monitoring of bone profile and vitamin D levels - prescribing additional vitamin D if	Consider Vitamin D and bisphosphonate treatment as recommended in DMD Care UK bone and endocrine guideline (26): http://tinyurl.com/k6dz2a5v

		turnover markers. However, vamorolone still increases risk of vertebral fractures compared to no-CS. Preliminary results however suggest that vertebral fractures are less common in boys treated with vamorolone compared to boys treated with daily prednisone or deflazacort (data up to 30-month treatment exposure). *Data presented but not peer reviewed to date* (28) There is currently no information on the impact of vamorolone on the risk of long bone fracture.	indicated (aiming for levels > 50 nmol/L). See DMD care UK bone and endocrine guideline (26): http://tinyurl.com/k6dz2a5v	

Behavioural issues	Cognitive and behavioural issues, including learning disabilities, autism spectrum disorders, attention-deficit hyperactivity disorder, anxiety, depression, are common in DMD. Behavioural problems, including anxiety, depression, temper tantrums, aggressivity, insomnia can be triggered or exacerbated by CS; but there is limited literature describing the impact of CS treatment on behaviour in DMD. Behavioural issues remain one of the main reasons behind treatment discontinuation.	Based on clinical experience, deflazacort might be associated with less behavioural problems than prednisolone; patients on intermittent regimens sometimes report greater fluctuations in their behaviour/mood than those on daily regimens. Vamorolone might be associated with less behavioural problems than prednisolone. *Data presented but not published to date (29).*	Consider psychosocial needs prior to treatment initiation and at each follow up appointment. Parents should be alerted about the possibility of deterioration of behaviour at the time of treatment initiation; in most cases, symptoms improve after a few weeks of treatment, but some behavioural difficulties can persist long term. Guidelines about behavioural assessment and management are currently in development by the DMD care UK Psychosocial working group.	Consider psychosocial assessment and support, including indication for pharmacological treatment, prior treatment initiation especially in children with pre-existing behavioural problems. A trial of evening dosing may be considered if symptoms disrupt school performance or attendance. Dose adjustments might be required if symptoms are distressing to the patient or the family.

Cushingoid features	Commonly observed. These features include round face and "buffalo hump" (excessive accumulation of fat between the shoulder blades) and can develop soon after treatment initiation although time of onset and severity can significantly vary from patient to patient. This side-effect can be distressing to patients, especially during adolescence.	Data are inconsistent comparing deflazacort and prednisolone. Some studies show higher frequency/worse severity with prednisolone, others with deflazacort. Intermittent regimens are reported to cause less Cushingoid features. Due to the inconsistent reporting of Cushingoid features with CS, it is difficult to compare vamorolone to other CS.	Physical examination at each follow up appointment. If significant Cushingoid features, consider the increased risk of sleep-disordered breathing. (20)	No treatment available. Dose adjustment can be considered if symptoms become distressing for the patient. However, the impact on muscle function, including respiratory function, should be carefully discussed before adjusting dose. Consider referral for psychological support.
Pubertal delay	Very common in patients on long term treatment with CS. It can affect bone health, growth and have a significant impact on mental health (self-esteem, confidence).	Clinical experience suggests that delayed puberty is present in most boys with DMD on daily CS and that this risk maybe lower in those on intermittent regimen. There is currently no information on the	Routine assessment of puberty. See DMD Care UK bone and endocrine guideline (26): http://tinyurl.com/k6dz2a5v	Testosterone therapy as appropriate. See DMD Care UK bone and endocrine guideline (26): http://tinyurl.com/k6dz2a5v

		impact of vamorolone on pubertal delay.		
Gastro-intestinal symptoms	These include epigastric pain, gastro-oesophageal reflux and peptic ulcer.	Unknown	Ask questions about symptoms such as gastric/abdominal pain and heartburn at each follow up appointment. Abdominal physical examination at each follow up visit if relevant.	Recommendation to take CS on full stomach (e.g. after breakfast). Avoid NSAIDs. Low threshold for proton-pump inhibitors (PPIs) and anti-acid in the presence of symptoms and/or clinical signs. In view of potential side effects with long term use of PPIs (e.g. reduced absorption of calcium and magnesium, vitamin B12 deficiency etc), PPIs are not recommended as prophylaxis. If long term PPIs are prescribed, monitor for vitamin D, vitamin B12 and magnesium levels and consider other side effects of long term PPI (e.g. Barrett's oesophagus).

				Consider further investigations and/or CS dose adjustments if symptoms persist.
Cataracts	Often asymptomatic.	The risk of developing cataracts is higher with deflazacort (reported 10% after three years on treatment) than prednisolone. There is currently limited information on the impact of vamorolone on the risk of developing cataracts.	Annual ophthalmology/ optician assessments.	Early signs of cataracts should trigger a referral to an ophthalmologist for monitoring. In most cases surgical treatment is not required as cataracts often remain asymptomatic. If removed, cataracts do not tend to re-occur.
Immunosuppression/increased risk of infections	CS are known to increase the white blood cell (WBC) count, predominantly due to increase number of circulating neutrophils (increased release from bone marrow and reduced apoptosis, without increase in neutrophil production). Although there is no clear evidence, patients with DMD on long term CS have an	Unknown	Ensure that chicken pox (varicella) and MMR immunisations are up to date (and tuberculosis immunisation for at risk populations) prior to CS initiation (with documentation of positive IgG following infection or complete vaccination) as they cannot be administered while on CS (live-vaccines).	Keep the national immunisation schedule, including pneumococcal 23 polyvalent, up to date. Avoid live vaccines while on treatment (attenuated vaccine to be used instead, e.g. IM flu jab instead of nasal formulation). Promptly treat infection.

	increased risk of community-acquired as well as nosocomial infection.		Advise parents and GPs on the risk of severe infection and importance to promptly address minor infections.	
Hypertrichosis	Usually mild and not distressing to the patient.	Unknown	Physical examination at each follow up appointment.	Inform parents. Usually not distressing for the patient; no interventions required.
Hypertension	Hypertension can occur with CS, both early after treatment initiation and after long term exposure.	Unknown	Check BP at each follow up appointment and compare to age and height appropriate centiles. Refer to the ESH guidelines for accurate monitoring of BP. (27)	Dietary advice on sodium intake and weight gain. Discuss with a cardiologist/hypertension clinic and consider treatment (ACE-inhibitors) if confirmed on repeated measurements. CS might need to be discontinued if hypertension is unresponsive to treatment.
Hyperglycaemia and diabetes	CS use is associated with abnormalities in glucose regulation and insulin sensitivity. Skeletal muscle is responsible for the majority of the post-prandial glucose uptake	Unknown	Monitor urine for glycosuria at each, 6-monthly follow up appointment. Dietary advice on managing sugar intake should be regularly reinforced.	If glycosuria is present, check for presence of ketones; a blood glucose should be performed at the time of detection of glycosuria. If blood glucose is 11.1 mmol/L or greater, or HbA1c 48 mol/mol or

	from the circulation and therefore abnormalities in glucose regulation could be more common in older adolescents and adults with DMD. Type 2 diabetes mellitus is not believed to be common but can occur.		Enquire with patients/families about polyuria/polydipsia. Further guidelines are currently in development by the DMD Care UK's nutrition working group.	grater, this is diagnostic of diabetes. Ask for symptoms of diabetes (polyuria, polydipsia, weight loss, lethargy) and discuss urgently with the diabetes team. Type 1 diabetes mellitus (a relatively common childhood chronic condition) should first be considered. If glycosuria is present and blood glucose is < 11.1 mmol/L , discuss with diabetes team who may advise further investigations (e.g. HbA1C or oral glucose tolerance test).
Skin fragility	CS induce atrophic changes in the skin that can lead to skin thinning and fragility, purpura, telangiectasia and stretch marks. Skin fragility can	Unknown	Physical examination at each follow up appointment.	Skin fragility requires close monitoring due to risk of infection. Wound healing must be carefully monitored.

	lead to slow wound healing. Purpura and telangiectasia can be more frequently observed on the face. Striae more commonly develop on the abdomen, hips, thighs and under the arms.			For striae, dietary advice for weight control. For the older, less mobile patients, carefully monitor for pressure ulcers and reinforce preventive measures.
Acne and other skin infections (e.g. tinea, warts)	Not frequently observed but can occur after long term exposure to CS. Acne may worsen during puberty).	Unknown	Physical examination at each follow up appointment. Inform the patient about the risk of developing acne around the teenage years.	For acne: use additional treatment measures (e.g. topical medications). Dose adjustments rarely needed. For other skin infections: use additional treatment measures (e.g. topical or oral medications). They can be difficult to treat, consider referral to dermatology. Dose adjustment might be considered in severe cases that do not respond to treatment.

Box 8: Importance of data-monitoring

With the recent approval of vamorolone as an additional corticosteroid option for DMD, the systematic collection of complete and accurate clinical data on the North Star database from all patients on any corticosteroid is even more critical. Such data collection will allow current gaps in knowledge to be addressed, particularly regarding the **longer-term** impact of vamorolone on muscle, respiratory and cardiac function and complications such as scoliosis. It will give a clearer picture of vamorolone's long-term, full side effect profile and how side effects and efficacy compare to classic corticosteroids. This will inform future standards of care and allow more informed decision making on the optimal corticosteroid type for individual patients.

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DMD Care UK is a collaborative initiative between the John Walton Muscular Dystrophy Research Centre at Newcastle University and Duchenne UK, embedded in the UK North Star Network. It is funded by Duchenne UK, Duchenne Research Fund and Joining Jack.