CORTICOSTEROIDS IN DUCHENNE MUSCULAR DYSTROPHY:

DMD Care UK standard of care guideline

March 31st 2025

Appendix A: Vamorolone – key considerations

Vamorolone is a corticosteroid (CS).

Please consult the Summary of Product Characteristics (SmPC) before prescribing vamorolone: https://www.medicines.org.uk/emc/product/15946/smpc

Vamorolone is approved in the UK for people with DMD aged 4 and older.

Vamorolone has only been studied as a daily treatment.

Vamorolone is only available in liquid formulation.

Vamorolone has shown similar effect to other CS on muscle function.

Vamorolone has shown a better side-effect profile compared to other CS with regard to growth and bone health, after up to 30-months of treatment.

Vamorolone has shown similar side effects related to weight gain and adrenal suppression.

Vamorolone might have a milder side effect profile with regard to behavioural problems.

There are no data on the longer-term safety and efficacy of vamorolone in DMD compared to other CS.

The impact on side effects of switching from long-term prednisolone or deflazacort to vamorolone is unknown. The possible effect of switching from intermittent regimens to daily vamorolone or from deflazacort to vamorolone on weight is unknown but should be discussed with families.

Similar to other CS, vamorolone should be taken in the morning, on a full stomach.

The recommended dose of vamorolone is 6 mg/kg/day. The recommended daily dose of vamorolone for patients with moderate hepatic impairment (Child-Pugh class B*) is 2 mg/kg/day (max dose of 80 mg for an individual with a body weight of 40 kg and above). Patients with severe hepatic impairment (Child-Pugh class C*) should not be treated with vamorolone.

*https://www.ncbi.nlm.nih.gov/books/NBK542308/

In the UK the maximum dose of vamorolone is 240 mg/day (equivalent to 6 ml). All individuals with a body weight > 40 kg can be prescribed a max of 240 mg of vamorolone/day.

Daily dose may be down-titrated to 4 mg/kg/day or 2 mg/kg/day based on individual tolerability. Patients should be maintained at the highest tolerated dose within the dose range.

Patients previously on classic glucocorticoids should switch to vamorolone 6 mg/kg/day to minimise the risk for adrenal crisis.

All individuals switching from prednisolone or deflazacort to vamorolone should watch for signs and symptoms of adrenal insufficiency (follow the Recommendations of the Endocrine & Bone Working Group of DMD Care UK: http://tinyurl.com/k6dz2a5v)

In contrast to other CS, vamorolone is a mineralocorticoid receptor antagonist (like spironolactone/eplerenone). Therefore, vamorolone should not be used for sick day dosing. Keep a low threshold to check electrolytes during acute illness, especially in individuals already on other mineralocorticoid receptor antagonists. The potential cardioprotective benefits of vamorolone still need to be investigated.

Based on the evidence available so far

Vamorolone might be preferred in young children, who could potentially benefit the most from the better safety profile with regard to growth and bone health.

Vamorolone might also be considered in people who were not previously prescribed with other CS either through family choice or concerns related to specific side effects. However, expectations need to be carefully managed given current lack of longer-term evidence of the side effect profile of vamorolone vs other CS.

Switching from prednisolone or deflazacort to vamorolone in older children should be evaluated on a case-by-case basis. The risks and benefits of treatment in this age group must be discussed with the individual and their carers, highlighting current uncertainties and the fact that data will increase in time.

Vamorolone has not been studied in adults with DMD, although some adults might now receive vamorolone in countries where it has been already approved. There are no published data on the effect of vamorolone in adults or on switching to vamorolone after several years on other CS. The benefit-risk of changing to vamorolone in this patient group should be carefully considered.

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