**CORTICOSTEROIDS IN DUCHENNE MUSCULAR DYSTROPHY: DMD Care UK standard of care guideline**

**Appendix D: Discussion about corticosteroids - LETTER TO A PATIENT’S GP**

I am writing to you as your patient, [*insert details*] has been prescribed/we are planning to prescribe your patient [*insert name*] with the corticosteroid prednisolone/deflazacort/vamorolone. (SELECT THE ONE THAT APPLIES) as part of their treatment for Duchenne muscular dystrophy (DMD).

Below you will find more information about this corticosteroid, its use in DMD and its side effects. However, they **key points** for you as this person’s GP are:

**Corticosteroids must not be discontinued suddenly as they cause *adrenal insufficiency* and therefore the risk of adrenal crisis. This can also be important during moderate/severe illnesses, surgery or physical traumas. In these situations, the child will require additional corticosteroids. GPs should:**

* Ensure continuation of the prescription.
* Ensure the family has written documentation of an emergency sick day plan (from the neuromuscular team).
* Supply the family with a stress dose of steroids at home for symptoms of adrenal insufficiency.

Before treatment starts:

* Ensure the child is up to date will vaccinations before CS is started.
* Establish varicella and measles immunity – arrange immunisation if necessary.
* Establish TB immunity and seek expert input if there is a TB risk

(DELETE ABOVE 3 POINTS IF ALREADY INITIATED)

Once treatment has been initiated:

* Do not use live vaccines once the CS treatment has started.
* Monitor blood pressure monthly for the first three months (risk of acute hypertension).
* Annually administer injectable influenza vaccine (do not give the live nasal vaccine).
* Give pneumococcal polysaccharide conjugate vaccines (Prevenar13).
* Encourage healthy diet and adequate intake of calcium and vitamin D.
* Avoid non-steroidal anti-inflammatory medication (NSAIDs). Use paracetamol as first analgesic option.
* Advise the family that corticosteroids should be taken on a full stomach at breakfast time or after breakfast.

**Further important details**

*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 DMD in people 4 years and over in January 2025. It now adds an additional option when considering corticosteroid treatment for DMD as part of the standard of care (if you would like further information about vamorolone in DMD, please find attached the summary of current evidence). TO BE ADDED ONLY IF PATIENT IS STARTED ON VAMOROLONE*

Corticosteroids are part of the standards of care for Duchenne muscular dystrophy (DMD). All corticosteroids however are associated with **side effects** which need to be carefully monitored and appropriately managed. Side effects can vary in their intensity and not every child develops all side effects.

Apparent within the first 6 months: adrenal insufficiency, increased appetite, epigastric pain and gastroesophageal reflux, behavioural changes (e.g. temper-tantrums; emotional behaviour; aggressiveness; insomnia), increased blood pressure and immunosuppression with increased risk of infection are the most common observed side effects.

In addition to the above and after longer-term exposure: Cushingoid appearance, stunting of growth, osteoporosis and increased risk of fractures, including vertebral fractures, delayed puberty, peptic ulcer disease, cataracts, insulin resistance, excessive hair growth and skin infections.

For more information about corticosteroid prescription in DMD, benefits and side effects, please refer to the DMD Care UK corticosteroid guidance (PROVIDE THE LINK). Moreover family guidance on corticosteroids in DMD is available and has been shared with the family (EITHER ENCLOSED OR PROVIDE THE LINK).

**REQUIRED MONITORING BY GPs**

*Prednisolone/deflazacort is usually prescribed by the GP. Vamorolone is prescribed by the neuromuscular specialist. (SELECT THE OPTION THAT APPLIES TO THE INDIVIDUAL CASE)*

Most of the monitoring of benefits and side effects of corticosteroids, and therefore dose adjustments, are performed by the specialist neuromuscular team. There are however actions for which the GP involvement is critical.

Before starting corticosteroids (TO BE INCLUDED IN LETTER SENT PRIOR TO STEROID INITIATION)

* Ensure the child is up to date with **vaccinations** according to national guidance. Live-vaccines are contra-indicated in children on long term treatment with high doses of corticosteroids. It is therefore important that all pre-school vaccinations are completed before treatment initiation.
* Establish varicella and measles immunity. If IgG antibodies are not detected, the child 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>).
* Assess for TB risk and establish immunity. Follow national guidance for those children at risk.

**Once corticosteroids are initiated:**

* GP to monitor blood pressure monthly for the first three months due to the risk of acute hypertension. Blood pressure monitoring will subsequently continue 6-monthly as part routine neuromuscular follow up.
* Annually administer injectable influenza vaccine (do not give the live nasal vaccine).
* Give pneumococcal polysaccharide conjugate vaccines (Prevenar13) as per national guidance (Childs et al., 2023 <https://pubmed.ncbi.nlm.nih.gov/38123347/>).
* Encourage healthy diet and adequate intake of calcium and vitamin D.
* Whenever possible, avoid non-steroidal anti-inflammatory medication (NSAIDs) such as ibuprofen and similar as they can worsen the irritation of the stomach lining. Paracetamol should be used as first analgesic option.
* Advise the family that corticosteroids should be taken on a full stomach at breakfast time or after breakfast, as at this time of the day helps mimic the body's own production of steroids and food provides protection for the stomach lining.

**Corticosteroids must not be discontinued suddenly as they cause *adrenal insufficiency* and therefore the risk of adrenal crisis. This can also be important during moderate/severe illnesses, surgery or physical traumas. In these situations, the child will require additional corticosteroids.**

* Ensure continuation of the prescription.
* Ensure the family has written documentation of an emergency sick day plan to be followed during acute illness, provided by the neuromuscular team.
* Supply the family with a stress dose of steroids at home for symptoms of adrenal insufficiency as recommended by the neuromuscular team.
* Refer to the DMD Care UK Endocrine guidance for management of adrenal insufficiency in DMD on long-term corticosteroids (<http://tinyurl.com/k6dz2a5v>).
* Arrange fast track access to local paediatric ward if this is not in place already. (DELETE IF NOT APPLICABLE IN YOUR SERVICE)



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