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

**Appendix C: Discussion about corticosteroids to FAMILIES**

***Below is a template letter that can be adapted for use by clinical teams with their paediatric patients and families.***

In clinic, we had a discussion about the use of corticosteroids in DMD, as we are planning to start……/have agreed to start…. *[patient name]* on prednisolone/deflazacort/vamorolone (SELECT THE ONE THAT APPLIES).

*Vamorolone is a more recent steroid designed to treat DMD (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). Several studies have shown that corticosteroids maintain or slow down the decline of muscle strength and function over a certain period of time, allowing the boys to walk for longer and maintain arm function for longer. Corticosteroids in DMD are used long term as they can also help with upper body and spine strength, and delay breathing and heart complications. It is recommended to start corticosteroids between the age of 4-6 years and early treatment (4-5 years) gives more benefits on muscle strength and function.

All corticosteroids however are associated with side effects which need to be carefully monitored and appropriately managed.

Children can show variable response to steroids both in terms of benefit on muscle function and side effects. Side effects can vary in their intensity and not every child develops all side effects.

Ensuring that the benefits are greater than the side effects is a key part of the care of children with DMD on corticosteroids. Before starting corticosteroids, we will arrange some tests to make sure that it is safe for your child to start treatment and will agree an appropriate follow up plan.

Please refer to the DMD Care UK corticosteroid guidance for families (EITHER – ENCLOSED OR PROVIDE THE LINK) which provides more information about corticosteroid prescription in DMD, benefits and side effects. The guidance has also been shared with your GP.

**Corticosteroid recommendations**

* Corticosteroids should be taken on a full stomach at breakfast time or after breakfast, as this time of the day helps mimic the body's own production of steroids, and food provides protection for the stomach lining.
* Corticosteroids cause adrenal insufficiency. When oral corticosteroids are taken for more than a month, they cause the adrenal glands to stop producing their own steroids and become “sleepy” (adrenal insufficiency). This can be important during moderate/severe illnesses, surgery or physical trauma. In these situations, your child will require additional corticosteroids. You will be provided with an emergency steroid plan that explains what to do in these situations and we’ll make sure you have a strategy for delivering the additional medications. Refer to the DMD Care UK Endocrine guidance for management of adrenal insufficiency in DMD on long-term corticosteroids (PROVIDE LINK and/or enclose).
* For the same reason, corticosteroid doses must not be missed, and it is important not run out of corticosteroids. The emergency steroid plan includes information about how to manage situations when your child is unable to take oral corticosteroids (e.g. in case of vomiting).
* It is important to avoid non-steroidal anti-inflammatory medication (NSAIDs) such as ibuprofen and similar as they can worsen the irritation of the stomach lining and cause pain.  Paracetamol should be used instead.
* Children receiving long term corticosteroids should not receive live vaccines. This is because their immune system might not react as well as expected. It is therefore important that all preschool vaccinations are completed before starting steroids and that children receive an annual flu jab as intramuscular injection (and NOT nasal spray). Immunity against chicken pox and measles must be confirmed before starting corticosteroids.

**What should you expect from your GP once your child is on corticosteroids?**

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

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 your GP’s involvement is critical. We list below key monitoring which should be performed by the GP – if your GP has any questions, they can contact the neuromuscular team (PROVIDE CONTACT DETAILS).

Before starting corticosteroids, the GP should: (USE IF APPLICABLE)

* Ensure the child is up to date with vaccinations according to national guidance.
* Establish varicella and measles immunity, according to the national guidance.
* Assess for TB risk according to national guidance and if there are concerns, refer to the specialist for advice.

Once corticosteroids are started:

* Monitor blood pressure monthly for the first three months due to the risk of acute hypertension (high blood pressure).
* Annually administer injectable influenza vaccine (your child must not receive the nasal vaccine as it is a live vaccine).
* Give pneumococcal polysaccharide conjugate vaccines (Prevenar13) as per national guidance.
* Ensure you have continuation of the prescription so that you don’t run out.
* Provide you with a stress dose of steroids at home for symptoms of adrenal insufficiency as recommended by the neuromuscular team.



***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.***

***www.dmdcareuk.org***