

DEFLAZACORT INFORMATION SHEET

WHAT IS DEFLAZACORT?

Deflazacort™ belongs to a group of medications called corticosteroids (often called steroids). Steroids are used for many different disorders. In Duchenne Muscular Dystrophy (DMD), steroids have shown to improve muscle strength when your child first begins the medication. Then it has been shown to slow down the progression (or effects) of the disease. *Deflazacort™* does not change the outcome of the disorder. The steroids used include Prednisone™ or *Deflazacort™* and they have been shown to:

- Prolong ambulation (the ability to walk).
- Delay wheelchair dependence. If your child can walk for a longer period of time, it may allow him to walk during his growth spurt, and this may reduce the development of a scoliosis and the need for scoliosis surgery (surgery to reduce a curve in the spine).
- Slow down the onset and progression of respiratory (lung) and cardiac (heart) problems.
- Slow down the loss of arm function.

IS DEFLAZACORT™ THE SAME AS PREDNISONE™?

Deflazacort™ is a steroid like Prednisone™. At the equivalent dosage, both drugs offer the same benefits. The dose for *Deflazacort™* is usually 0.9 milligrams/kilogram of body weight/day while the dose for Prednisone™ is usually 0.75 mg/kg/day. *Deflazacort™* was developed with the hope of having fewer side effects than Prednisone™, such as weight gain. Some of the long-term side effects may also be decreased, but this has not yet been proven in well designed human studies.

WHAT ARE THE SIDE EFFECTS OF DEFLAZACORT?

Deflazacort™ is a steroid - a form of hormone. It has a powerful effect on the body and it may cause unwanted side effects. Most of these side effects are reversible. Some of these side effects could be serious, while others may be unpleasant but are not harmful. You and your child's doctor will decide together whether the benefits of *Deflazacort™* outweigh the risk of side effects.

- Keep appointments and discuss any concerns with the doctor. This allows her/him to adjust the dose to manage the condition with the least possible unwanted effects.
- Ask the clinic nurse, doctor or pharmacist for suggestions on ways to deal with side effects.

Potential Side Effects:

The most common side effects of steroids include weight gain and mood changes. Here are the side effects that can occur while taking *Deflazacort™*.

- Weight gain (due to increased appetite). Weight gain is most likely to happen when your child starts the medication.
- Mood changes.
- Gastro-intestinal upset (nausea). Take each dose with food to decrease nausea.
- Headaches.
- Difficulty sleeping.
- Roundness of the face (Cushing's features).
- Suppression of growth (short stature or height). This can be a benefit, because with DMD the muscles weaken during growth spurts.
- Loss of bone calcium – osteoporosis – this may increase the risk of broken bones. We try to minimize this with Calcium and Vitamin D supplements. Bone Density studies will be done yearly.
- Development of cataracts, although these do not usually impair your child's vision.
- Excessive hair growth.
- Development of acne.

(continued on reverse)

Other Potential Side Effects Can Include:

- Impaired (slow) wound healing.
- Increased susceptibility to infections.
- Mood changes which can include hyperactivity, irritability, confusion, hallucinations and depression.
- Dizziness.
- High blood pressure
- Diabetes Mellitus.

Contact your physician if you notice any of the following side effects:

- Severe stomach pain.
- Black stools (poop).
- Increased thirst and urination (peeing more often than usual).
- Convulsions (rare).

It is important to inform the doctor if any of these side effects are sudden or severe. The majority of these side effects are reversible when your child's medication is tapered off slowly. **Do not discontinue (have your child stop taking) Deflazacort™ without the consent and supervision of your child's doctor.**

IF MY CHILD HAS NEVER BEEN ON STEROIDS AND IS NOW A FULL TIME WHEELCHAIR USER, COULD HE GO ON STEROIDS?

Yes, as it may help maintain

- Upper limb function
- Respiratory (lung) function
- Cardiac (heart) function

IF MY CHILD STOPS WALKING, IS IT TIME TO STOP TAKING DEFLAZACORT?

Deflazacort does more than just help your child walk for a longer period of time. It appears to help the muscle function in his arms and delays onset of respiratory and cardiac problems. Thus we feel that it is worthwhile continuing on steroids even after your child stops walking. However, this will be decided individually with you and your child's doctor.

WHERE CAN I FIND OUT MORE ABOUT DEFLAZACORT?

1. Muscular Dystrophy Canada at www.muscle.ca
2. Muscular Dystrophy Association USA at www.mdausa.org. Note that this is an American website where care and treatment may differ from Canada.
3. Parent Project Muscular Dystrophy at www.parentprojectmd.org. Note that this is an American website where care and treatment may differ from Canada.
4. Biggar WD, Gingras M, Fehlings DL, et al., Deflazacort Treatment of Duchenne Muscular Dystrophy. *Journal of Pediatrics* 2001; 138: pp 45-50.
5. Dubowitz V, Kinali M, Main M, et al., Remission of Clinical Signs in Early Duchenne Dystrophy on Intermittent Low-Dosage Prednisone Therapy. *Ger. J. Paediatr Neurology* 2002; 6: pp 153-159.
6. Bonifate MD, Ruzza G, Bonometto P, et al., A Multicentre, Double-Blind Randomized Trial of Deflazacort vs. Prednisone in Duchenne Dystrophy. *Muscle Nerve* 2000; 23: pp 1344-1347.

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