Corticosteroids and DBA

A corticosteroid is a powerful drug that is commonly used to treat many conditions including allergic reactions and inflammation. Although it is not fully understood, corticosteroids also help some DBA patients make more red blood cells. For those patients responding to steroids, the goal is to maintain a hemoglobin level of 9 g/dL or higher without the need for transfusions. When considering the initiation of steroid treatment (often recommended after 1 year of age), it is important to discuss the basic guidelines of this therapy with your physician, taking under consideration the patient's current hematologic status and overall well-being. The main goal in using steroid therapy is to increase red blood cell production while balancing the side effects typical of steroid therapy.

The initial treatment is typically a starting dose of 2 mg/kg/day of prednisone or prednisolone for a trial period of several weeks. With a response to steroids resulting in a rise in hemoglobin, the physician will SLOWLY and carefully lower the initial dose. Over the course of several weeks and months, hemoglobin levels will be monitored and the steroid dose will be adjusted accordingly. **Currently the recommended target maintenance dose should not be more than 0.5 mg/kg every day or 1 mg/kg every other day.**

Pregnancy and increased growth may warrant an adjustment in steroid dose, and for reasons that are not fully understood, some patients experience unexpected declines in hemoglobin levels and require adjustments to their dose. Colds and other infections may also result in dips in hemoglobin which can be temporary or permanent. Some people experience automatic recovery while others require dose adjustment to increase red blood cell production. Others may requires a blood transfusion to return their hemoglobin to a safe level while waiting for the boosted steroid dose to take effect.

Every DBA patient is different to their response to steroid therapy. Some patients respond to low, seemingly homeopathic doses, some patients have no response, some patients respond only at high doses, and others initially respond but later become refractory. Remission is a possibility for patients on steroids and those on blood transfusion therapy.

Side effects, both short and long-term, are common when taking steroids. It is necessary to be aware, vigilant, and proactive to avoid or minimize the side effects. A low dose of corticosteroids may be adequate to maintain a normal hemoglobin level with few side effects. High doses over a prolonged period can cause serious side effects. All patients taking steroids should be carefully monitored. Your physician may suggest other treatment options if serious side effects develop.

<u>Possible side effects of short-term use:</u>

- upset stomach
- increased blood sugar
- increased hunger
- behavior changes, trouble sleeping, irritability
- increased risk of pneumonia, thrush (white coating in the mouth) and other infections
- weight gain, salt and water retention
- high blood pressure
- increased fat on the face (rounded face), upper back and belly
- stretch marks on the skin, acne, poor wound healing, increased and unusual hair growth

<u>Possible side effects of long-term use (3 months or longer):</u>

- all short-term side effects
- poor growth in children (can be severe)
- osteoporosis (bones break easily, problems with hips and shoulder joints)
- muscle weakness
- diabetes
- increased risk of developing cataracts
- increased risk of developing stomach ulcers
- the adrenal glands may shut off, making it impossible to produce cortisol during emergencies (it may be necessary to 'stress dose' during these times).

Patients who take steroids should be aware of other necessary precautions. Certain medications may be taken to avoid or treat specific side effects, and your doctor may suggest additional monitoring while on steroid therapy. While on corticosteroid therapy, patients should call their doctors if they have a fever of 100.5°F more than once, or a fever of 101.0 °F even once. Talk to your doctor about your concerns including stomach issues, growth retardation and bone health. Following these medical recommendations and taking care of yourself will help to ensure that you are doing all you can to prevent the pitfalls that come with steroid therapy. It may be required to take hydrocortisone if your doctor determines steroid therapy will be discontinued. This is due to the adrenal glands not producing cortisol after depending on the steroids for so long. This can be determined by measuring an am cortisol level.

Other medications:

- Antibiotics – a prophylactic dose of an antibiotic can help ensure that the patient does not contract certain types of pneumonia while taking high doses of steroids for long periods of time and if white blood counts are low at start of therapy.
- Anti-fungal – may be helpful in treating yeast-based diaper rash or thrush and should only be taken as needed
- Acid reflux medication – may help in relieving and sometimes preventing stomach problems
- Vaccines – patients on steroids should speak with their doctor about their vaccine schedule. Often, live vaccines are avoided. Re-vaccination may be necessary if steroids were started before completion of the initial series of immunization.

Recommended on-going medical care:

- Monitor CBC monthly (or less frequently) once a steady dose is attained
- Measure height routinely. A 'steroid break' may be needed if growth has fallen below the growth curve for age, especially during the first year of life and during puberty
- Annual vision check
- Dental visits twice a year
- Baseline dexascan (x-ray to determine the strength of the bones) and follow-up scans as needed to monitor bone loss
- Endocrinology referral - This doctor can be helpful with growth and development issues as well as discussing side effects from short term steroid usage and long-term use.
- Yearly flu shot (not the nose spray vaccine)
- Try to avoid anyone who has viral infections.
- Careful monitoring should be considered during puberty. Both the hormonal and physical changes that take place during this time can affect DBA patients and his/her treatment.
- Women who become pregnant while on steroids should be carefully monitored. It may be necessary to go to transfusion therapy during pregnancy. It is important to include your hematologist in family-planning discussions prior to becoming pregnant if at all possible.
- You cannot abruptly stop steroid therapy. You must be weaned over a period of time as you can experience steroid withdrawal.

Some people on long-term steroid therapy wear a bracelet or necklace engraved with the patient's medical and personal information to ensure that the response team will be able to provide proper treatment in the event you are unable to communicate.

DBA patients taking corticosteroids can testify that this treatment plan has the benefits of being transfusion independent, but is not without other side effects and risks. Working closely with your team of physicians to meet recommended treatment guidelines and obtain appropriate on-going medical care help make steroid treatment therapy an option for living with DBA.