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Drug Therapy

Version of 2016

5. Azathioprine

5.1 Description

Azathioprine is an immunosuppressive medication.

It works by interfering with the production of DNA, a process that all cells need to undergo in order to divide. The inhibition of the immune function is in fact due to the effects of the drug on the growth of one kind of white cell of the blood (lymphocytes).

5.2 Dosage/modes of administration

It is administered orally at a dosage of 2-3 mg per kg per day, up to a maximum of 150 mg per day.

5.3 Side effects

Although usually better tolerated than cyclophosphamide, azathioprine can have some side effects that need close monitoring. Toxicity to the gastrointestinal tract (oral ulcers, nausea, vomiting, diarrhoea, epigastric pain) is uncommon. Liver toxicity may occur but is rare. A reduction in the number of circulating white blood cells (leukopenia) may occur and it is in most cases dose-related; less common is the reduction in the number of platelets or red blood cells. Around 10% of patients are at higher risk of haematological complications (cytopenia, or a decrease in white blood cells, red blood cells or platelets) due to a possible genetic defect (partial thiopurine methyltransferase -TPMT- deficiency also known as genetic polymorphism). This can be tested for before starting the treatment and the control of blood cell counts can

be performed 7 to 10 days after treatment onset and then at regular monthly or bi-monthly intervals.

The long-term use of azathioprine may theoretically be associated with an increased risk of cancer but so far the evidence is not conclusive. As with other immunosuppressive agents, treatment exposes the patient to an increased risk of infections; herpes zoster infection in particular is observed with higher frequency in patients treated with azathioprine.

5.4 Main paediatric rheumatic diseases indications

Juvenile systemic lupus erythematosus.

Some paediatric systemic vasculitis.