Rituximab for treating immune (idiopathic) thrombocytopenic purpura

Information for the public
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About this information

This information explains the evidence summary about the off-label use of rituximab for treating immune (idiopathic) thrombocytopenic purpura (known as ITP for short). The evidence summary is an overview of the available information about this medicine. It aims to help prescribers and patients when they are considering whether or not to use an unlicensed or off-label treatment. The summary does not contain recommendations from NICE on whether the medicine should be used.

Licensing medicines

In the UK, medicines need to have a licence before they can be marketed. To get a licence, the manufacturer of the medicine has to provide evidence that shows that the medicine works well enough and is safe enough to be used for a specific condition and for a specific group of patients, and that they can manufacture the medicine to the required quality.

Medicines can be prescribed without a licence (an 'unlicensed medicine') if there is no suitable licensed alternative and it is likely to benefit the patient.

A medicine can also be prescribed 'off-label'. This means the prescriber wants to use it in a different way than is set out in the terms of its licence. This could mean using the medicine for a different condition or a different group of patients, or it could mean a change in the dose or that the medicine is taken in a different way. There is more information about licensing medicines on NHS Choices.

What is immune (idiopathic) thrombocytopenic purpura?

Immune (idiopathic) thrombocytopenic purpura (or ITP for short) is a bleeding disorder caused by a shortage of tiny cells in the blood called platelets. When a person has an injury such as a cut to the skin, platelets help the blood to form a clot and stop the bleeding. People with ITP have fewer platelets and an increased risk of bleeding which, in rare cases, may be life-threatening.

In adults, ITP is usually a long-term condition. Many adults with ITP do not need any treatment, unless the number of platelets they have in their blood falls below a certain number, they have significant bleeding symptoms or they need to have surgery for any reason (including dental work). If treatment is needed, then the first treatments that are used are usually steroids or a medicine called immunoglobulin. These medicines help to increase the number of platelets in a person's blood. Sometimes, a person might have to have surgery to remove their spleen (called a splenectomy), but this might not be suitable for everybody. Other treatments that might be used include medicines called azathioprine, ciclosporin, cyclophosphamide, dapsone, mycophenolate mofetil, rituximab, eltrombopag, romiplostim and vinca alkaloids.

Most children who have just been diagnosed with ITP do not need any treatment, and the condition usually goes away in 6–8 weeks. However, children with more severe bleeding symptoms, or children at an increased risk of bleeding usually need some type of treatment. The first treatments that are tried tend to be the same as those for adults.

There are also other medicines that a doctor might try to treat ITP in children and young people– these tend to vary depending on the person and their condition. Occasionally, surgery to remove the spleen (a splenectomy) might be suggested.

About rituximab

Rituximab is a medicine known as a 'monoclonal antibody'.

It is licensed for treating various conditions in adults (aged 18 years and over) including non-Hodgkin's lymphoma, chronic lymphocytic leukaemia, rheumatoid arthritis, and granulomatosis with polyangiitis, or microscopic polyangiitis. It is not licensed for use in children. Rituximab is given by a drip (also known as an infusion) into a vein. This would usually be carried out in hospital by a doctor or nurse who has experience in using the treatment.

Rituximab is not licensed for treating ITP in children or adults, and so using it for ITP is 'off-label'.

Summary of possible benefits and harms

How well does rituximab work?

It is difficult to say how well rituximab works for treating ITP in children, young people and adults.

Some of the studies in adults showed that rituximab helped to increase the number of platelets in a person's blood. Most of the studies didn't compare rituximab with other treatments for ITP so it is hard to say how well rituximab worked compared with other treatments. Although, there were some studies that compared how well rituximab worked with other treatments or a dummy treatment (a treatment that doesn't contain any medicine, also known as a placebo), the number of people in the studies were small and the studies were not well designed making it difficult to say how well rituximab works for treating ITP compared with other treatments.

Some of the studies in children and young people also showed that rituximab helped to increase the number of platelets in a person's blood. The studies didn't compare rituximab with any other treatments for ITP and were not well designed. This makes is difficult to say how well rituximab works in children and young people with ITP.

Studies that are of better quality and have more people in them are needed to be able to

say how well rituximab works for treating ITP, especially in children and young people.

What are the possible harms or side effects?

Out of 10 people who have treatment with rituximab through a drip, more than 1 can have a reaction to it; this usually happens during or within the first 2 hours of treatment. The reaction might include fever, chills and shivering. Less often, some people might get reactions including pain where the drip is put in, blisters, itching, sickness, tiredness, headache, breathing difficulties, swelling in the tongue or throat, an itchy or runny nose, vomiting, flushing or palpitations, a heart attack, or a low number of platelets. If people get any of these symptoms, the drip might need to be slowed down or stopped or they might need to take an antihistamine or paracetamol. These reactions are more likely to happen the first time a person is treated with rituximab and are less likely to happen after further treatments. However the person's doctor may decide to stop treatment if the reactions are serious.

People who are receiving rituximab can get infections more easily during or after treatment with it. These infections will often be minor, for example, a person may get a viral infection such as a cold after being treated, but there have been cases of more severe infections such as pneumonia or urinary infections. Rituximab shouldn't be used by people who already have a severe infection, or by people who have hepatitis B.

Rituximab can cause a serious infection of the brain (called progressive multifocal leukoencephalopathy or PML) which can be fatal, but getting this type of infection is very rare. If a person has memory loss, trouble thinking, difficulty walking or sight loss during treatment with rituximab, they should tell their doctor immediately. Rituximab can also very rarely cause skin reactions including severe blistering skin conditions that can be life-threatening. Redness (often with blisters) may appear on the skin or on mucous membranes (such as inside the mouth, the genital areas or the eyelids) and sometimes people get a fever. People should talk to their doctor immediately if they have any of these symptoms.

All people who are being treated for rheumatoid arthritis, granulomatosis with polyangiitis and microscopic polyangiitis must be given a 'patient alert card' each time they are given rituximab. The alert card contains important safety information.

Please note that the results of the research studies only indicate the benefits and harms for the population in the studies. It is not possible to predict what the benefits and harms will be for an individual patient being treated with rituximab.

Prescribing rituximab

If a prescriber wants to use an unlicensed or off-label medicine, they must follow their professional guide, for example for doctors the General Medical Council's good practice guidelines. These include giving information about the treatment and discussing the possible benefits and harms so that the patient has enough information to decide whether or not to have the treatment. This is called giving informed consent.

A full version of the summary aimed at healthcare professionals is available on the NICE website. The summary for healthcare professionals does not contain recommendations from NICE on whether the medicine should be used.

Questions to ask

- Why am I or my child being offered an off-label medicine?
- What does the treatment involve?
- What are the benefits I or my child might get?
- How good are my chances of getting those benefits?
- Could having the treatment make me or my child feel worse?
- Are there alternative treatments?
- What are the risks of the treatment?
- Are the risks minor or serious? How likely are they to happen?
- What may happen if I or my child don't have the treatment?

More information

NICE has published information about how evidence summaries for unlicensed and off-label medicines are developed.

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