NICE National Institute for Health and Care Excellence

NICE information for the public

Rituximab for treating autoimmune haemolytic anaemia

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 autoimmune haemolytic anaemia. 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 autoimmune haemolytic anaemia?

Blood is made up of a liquid (called plasma) and various types of cells including red blood cells. Red blood cells contain a substance called haemoglobin, which carries oxygen around the body to the tissues. When the level of haemoglobin is low a person is short of oxygen and feels tired. This is called anaemia.

Autoimmune haemolytic anaemia is a type of anaemia that develops when the antibodies (types of protein) of a person's immune system damage some of their red blood cells.

Autoimmune haemolytic anaemia is normally divided into 2 types, depending on the type of antibody. These are warm antibody type (the most common form) and cold antibody type. Treatment depends on the type of antibody causing the anaemia.

Not all people with warm type autoimmune haemolytic anaemia will need treatment, but for those who do, the first treatment is usually steroids. Other medicines which work on the immune system or rituximab might be used after steroids have been tried. Another option is surgery to remove the spleen (called a splenectomy).

Steroids, medicines which work on the immune system or surgery to remove the spleen don't usually work very well in cold type autoimmune haemolytic anaemia. People with cold type autoimmune haemolytic anaemia are now often offered rituximab as the first treatment.

About rituximab

Rituximab is a medicine that can help to stop a person's antibodies from destroying their own red blood cells.

Rituximab is licensed for treating various conditions in adults (aged 18 years and over). These include 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, usually once a week for 4 weeks for autoimmune haemolytic anaemia. This is usually done in hospital by a doctor or nurse who has experience in using the treatment. Usually only 1 course of treatment is required, although repeat courses are sometimes used, and sometimes rituximab is used in combination with steroids or other medicines.

Rituximab is not licensed for treating autoimmune haemolytic anaemia in children or adults, and so using it for this condition is 'off-label'.

Summary of possible benefits and harms

How well does rituximab work?

One high quality study looked at how well rituximab worked for treating warm type autoimmune haemolytic anaemia in adults who hadn't received any past treatment for the condition. The study found that 12 months' treatment with prednisolone (a steroid) and rituximab was better at increasing a person's haemoglobin to a normal level than prednisolone alone.

Other studies in children, young people and adults with warm type autoimmune haemolytic anaemia, and in adults with cold type autoimmune haemolytic anaemia, showed that rituximab increased a person's haemoglobin level. However, the studies were not well designed and they didn't compare rituximab with other treatments, making it difficult to be very sure how well rituximab will work for someone with autoimmune haemolytic anaemia.

What are the possible harms or side effects?

In the high quality study that looked at how well rituximab and a steroid worked for treating warm type autoimmune haemolytic anaemia in adults with no past treatment for the condition, the most common side effects were breathlessness, tiredness, headache, indigestion and difficulty sleeping (several of which were probably related to the steroid).

Side effects that have occurred in people who take rituximab for the conditions for which it is licensed are discussed below.

For every 10 people who are given 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 (cells in the blood that help the blood to clot). If people do get any of these symptoms during the treatment, 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 given rituximab and are less likely to happen after further treatments. However, the person and their doctor may decide to stop treatment if the reactions are serious.

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

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

Everyone who is being treated with rituximab 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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