

# Rituximab for treating minimal change disease and focal segmental glomerulosclerosis

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 minimal change disease and focal segmental glomerulosclerosis. 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 are minimal change disease and focal segmental glomerulosclerosis?

Minimal change disease and focal segmental glomerulosclerosis are conditions that can affect the kidneys. They are a form of glomerular disease, with damage to the tiny structures within the kidneys (called the glomeruli) which filter the blood to make urine. Damage to the glomeruli can affect how well the kidneys work. Protein from the blood can leak into the urine. This can cause swelling of the feet and legs, and sometimes rapid build-up of fluid in other parts of the body. Other complications include high cholesterol and blood clots. Medicines are prescribed to manage these complications.

In minimal change disease, treatment aims to control the condition and stop further damage to the kidneys. In the first instance, steroids are given at high doses. These are reduced slowly and then stopped in many cases. In some cases, the condition comes back when the dose of steroid is reduced. This is called steroid-dependent minimal change disease. Sometimes larger doses of steroids are needed to control the condition or steroids don't seem to work very well at all. This is known as steroid-resistant minimal change disease. In these situations, a specialist doctor may prescribe different medicines with or without the steroids. These other medicines include cyclophosphamide, ciclosporin, tacrolimus, mycophenolate mofetil and rituximab.

In focal segmental glomerulosclerosis, treatment aims to control the condition and slow down the rate of kidney damage. The medicines used for focal segmental glomerulosclerosis are the same as for minimal change disease. As in minimal change disease, focal segmental glomerulosclerosis can be steroid resistant or steroid dependent.

## About rituximab

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.

Rituximab is given by a drip (also known as an infusion) into a vein. 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 needed, although sometimes repeat courses are given, and sometimes rituximab is used with steroids or other medicines.

Rituximab is not licensed for treating minimal change disease or focal segmental glomerulosclerosis in adults and so using it for these conditions 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 minimal change disease and focal segmental glomerulosclerosis in adults because the studies were small and did not have good comparison groups. Also, the amount of rituximab given was different and some people were also taking other medicines such as steroids.

Some studies showed that rituximab helped to reduce the number of times the condition flared up within a year and the amount of protein leaking into the urine. Some people were able to reduce or stop other medicines such as steroids and those that suppress the immune system when rituximab was given. These studies did not compare rituximab with other treatments for minimal change disease or focal segmental glomerulosclerosis, so it is hard to say how well rituximab worked compared with other treatments. One small study in adults whose condition had come back after a kidney transplant compared people taking rituximab with people taking other treatments. Rituximab was no better than other treatments at improving minimal change disease or focal segmental glomerulosclerosis.

### What are the possible harms or side effects?

Rituximab has been used for a number of years and in a lot of people for other conditions

so the side effects are well known. In the studies that looked at how well rituximab worked for treating minimal change disease and focal segmental glomerulosclerosis in adults, the most common side effects were related to the infusion of rituximab. Side effects included unexpected low blood pressure that lasted for a short time, itchy red eyes, cough, hiccup and a skin rash. One person had inflammation in the lungs (bronchopneumonia) 2 months after treatment with rituximab. One study reported a long-term complication of slightly reduced white cells (also called mild leukopenia).

Side effects that have occurred in people taking 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, usually within the first 2 hours. This might include fever, chills and shivering. Less often, people have 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, sickness, flushing or palpitations, a heart attack, or a low number of platelets (blood cells that help the blood to clot). If people have any of these symptoms, the drip might need to be slowed down or stopped. Paracetamol and an antihistamine are given before each dose of rituximab to manage some of these symptoms. These reactions are more likely the first time rituximab is given but 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 are often minor (for example, a viral infection such as a cold) but there have been cases of more severe infections such as pneumonia and urinary infections. Rituximab shouldn't be given to people who already have a severe infection, or to people with hepatitis B. People will be checked for this before they are prescribed rituximab.

Rituximab can cause a serious infection of the brain (called progressive multifocal leukoencephalopathy or PML) which can be fatal. 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 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.

Please note that the results of the research study only indicate the benefits and harms for the population in the study. 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 being offered an unlicensed medicine?
- What does the treatment involve?
- What are the benefits I might get?
- How good are my chances of getting those benefits?
- Could having the treatment make me 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 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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