

# Sirolimus for the treatment of children with vascular anomalies

Information for parents and carers



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This leaflet provides information on a course of treatment called sirolimus. The leaflet will explain what this is and when and how it will be given.

It will also tell you about common side-effects that your child may experience. Contact telephone numbers and details of how to obtain further information are given at the end of the leaflet. Before your child starts this treatment, read the manufacturer's printed information leaflet from inside the pack. It will give you more information about sirolimus and will provide you with a full list of side-effects which you may experience from taking it.

## **What is sirolimus?**

Sirolimus is an immunosuppressant medicine, which dampens down the immune system. It is most commonly used following kidney transplant to prevent the new kidney being rejected by the body. However it is now being used for other medical conditions.

## **Why is my child being offered sirolimus?**

Sirolimus is used to try and reduce the size and effect of the lymphatic malformation to reduce pain and disability if the underlying anomaly is leading to any functional problem.

Your doctor has prescribed this treatment because it is believed to be effective in treating your child's type of vascular anomaly.

However, it is currently unlicensed for use in children and the use of sirolimus for vascular anomalies is also outside of its product licence. You can be assured that your doctor has only prescribed an 'unlicensed' medicine because he or she thinks that the medicine will benefit your child and no licensed alternative is available.

### What is an unlicensed drug?

In the UK, all medicines undergo a process of checks to ensure that they are safe and effective. The licence (also known as the 'Marketing Authorisation') for a medicine will be specific for treating a particular condition or range of conditions. When a medication is used for a condition not stated in its licence, it is said to be 'unlicensed'. This is actually very common in paediatric practice (children's medicine) because a lot of medications are only tested with adults. In the case of sirolimus, your doctor has prescribed an unlicensed medication for you because they are confident that this medicine will work well for your condition even though it is not licensed for this condition. If you would like to know more about unlicensed medications in children you can go to [www.medicinesforchildren.org.uk/unlicensed-medicines](http://www.medicinesforchildren.org.uk/unlicensed-medicines) or speak to your consultant.

### How long will my child be offered sirolimus?

Normally, we offer sirolimus for two years, after which we arrange scans of your child's malformation. This would most likely be an MRI. We will stop sirolimus earlier if your child is experiencing side-effects.

## Who should not use sirolimus?

- Hypersensitivity to sirolimus or any of its ingredients – it is not suitable for people allergic to soya or peanuts.
- Pregnant, could be pregnant, planning to become pregnant or breastfeeding.
- Existing liver problems.
- Low immune system.

## How is sirolimus monitored?

Several tests need to be carried out both before starting and while on treatment. These help your doctor work out if it is both safe to start and continue treatment. Some tests also provide your doctor with baseline information so that they can later assess if the treatment is working.

### Blood tests

#### *Children have the following tests before starting sirolimus:*

- Urea and electrolytes
- Liver function
- Full blood count
- Clotting profile
- Fasting cholesterol and triglycerides
- Immunoglobulins
- Immune response to certain vaccines such as tetanus, pneumococcus, measles
- Immunity to chicken pox

- Tests for certain viruses such as HIV, hepatitis B and C, CMV and EBV.

If any of these results are abnormal, your doctor will discuss what needs to be done.

***Once treatment has been started, the following blood tests will be needed:***

- Sirolimus level
- Urea and electrolytes
- Liver function
- Full blood counts
- Clotting profile
- Fasting cholesterol and triglycerides

The sirolimus level needs to be taken just before the dose is due to be given. Therefore levels are usually checked in the morning and your doctor will ask you NOT to give the dose of sirolimus that morning until after the bloods have been taken.

As fasting cholesterol and triglyceride levels need to be checked, your child should not have anything to eat or drink from midnight before the blood test.

Blood tests will be need to be repeated every seven days until the dose of sirolimus has been adjusted for your child. After this, tests will be required monthly and then three monthly. If the dose or formulation changes, these blood tests will need to be repeated seven days after the change has been introduced. Your doctor will explain this to you in more detail.

## Scans

Before starting sirolimus, your child may need an MRI of the area affected by their condition (usually with injected contrast) and a whole body MRI. This provides your doctor with more information and acts as a baseline before starting treatment. A repeat MRI scan may be needed approximately one year or two years after starting treatment but this will again be discussed by your doctor.

## How should my child take sirolimus?

Sirolimus should be taken once a day, at the same time each day. The dose of sirolimus should be given consistently, either with food or without food to ensure that the same amount of active ingredient is absorbed by the body with every dose. We recommend they should take their dose in the morning, within 30 minutes of finishing their meal. It is important that you and your child wash your hands thoroughly after giving and taking sirolimus. If your child is taking tablets they should be swallowed whole with a glass of water. **Tablets must not be crushed or chewed.**

**If your child is taking oral solution follow the directions below.**

### How should my child take sirolimus oral solution?

The manufacturer recommends that each dose be diluted – instructions are given below.

#### *If you are diluting the medicine, you will need:*

- The bottle of sirolimus 1mg/1ml oral solution.
- Syringe adapter.
- Oral syringe with dose marked on it.

- Glass or plastic cup.
- Water or orange juice – **do not use any other liquid to mix the dose.**



**Directions:**

1. Wash your hands with soap and water.
2. Shake the bottle and remove the top and put to one side.
3. Insert the syringe adapter into the top of the bottle – leave this in the bottle when you have given the dose.
4. Draw up the dose of sirolimus in the syringe provided.
5. Pour half the volume of water or orange juice into a glass or plastic cup.
6. Empty the syringe into the water or orange juice and mix thoroughly.
7. Give to your child to drink.
8. Add of the rest of the volume of water or orange juice to the cup and mix thoroughly to pick up any remaining sirolimus oral solution.
9. Give to your child to drink.
10. Put the top back on the bottle.

Dispose of the used syringe as you have been taught and wash the cup in warm, soapy water. If you need to give the dose later in the day, you can store the filled syringe for up to 24 hours at room temperature. When it is mixed with water or orange juice, you should give it to your child immediately.

**Please discuss the volume of water or orange juice to use with your doctor.** However if your child cannot tolerate this volume, it can be given undiluted but the thickness and taste of the medicine can make it difficult to give. If given undiluted, we recommend giving your child a drink of water afterwards to reduce the risk of mouth ulcers.

## How much sirolimus will they need to take?

This will vary for each person and the amount of sirolimus your child takes may change over time. The amount of sirolimus they need to take is based on blood tests to check the level of the drug in their blood. Your consultant will explain how much they need take based on these blood tests.

## How often will my child see the specialist team?

When they start treatment they will see the specialist team each week. Your child will have blood tests every week and we will check how they are feeling and discuss any problems they may have.

Depending on your child's blood results we will tell you if they need to change the amount of sirolimus they take. They will have blood tests every week to start with. Then less often if the drug levels in their blood are stable.

It is important that the blood test is taken before your child takes that day's dose. If you child takes their sirolimus in the morning, then do not give them a dose on the morning of their blood test but bring the dose with you so they can take it afterwards.

## How should I store the tablets?

Tablets should be stored in their original packaging in a safe place away from children. They should be kept in a cool dry place. Any unused tablets should be returned to the hospital pharmacy or your local chemist for safe disposal.

## How should I store the liquid?

Sirolimus 1mg/1ml oral solution comes in a bottle and is a yellow colour. The oral solution sometimes develops a cloudy appearance. This is not harmful and will disappear when the bottle is brought up to room temperature.

Sirolimus liquid should be stored in its original packaging, in a refrigerator, in a safe space and away from children. A dose can be stored in the syringe for 24 hours at room temperature but must be given immediately once mixed with juice or water.

## What should I do if my child forgets to take their sirolimus?

They should take their normal dose the following day. Do not take a double dose to make up for the forgotten dose.

## What if I my child takes too much sirolimus?

If your child takes too many tablets or too much oral solution than prescribed, please seek urgent medical attention via A&E.

## Allergies

Sirolimus oral solution contains soya oil, *patients with an allergy to peanuts and/or soya must not take this medicine.*

## What are the possible side-effects?

There are a number of possible side effects which can occur with this treatment. The doctors, nurses and pharmacists can give you advice or answer any questions you may have.

### *Common side-effects affecting about 1 in 10 patients include:*

- Headache
- Fast heart rate (tachycardia)
- Tiredness and fatigue
- nausea, stomach pain, diarrhoea or constipation
- Sore mouth and ulcers. If this affects your child's ability to eat or drink contact your medical team for advice
- Infections: as sirolimus dampens down the immune system your child is more likely to get mouth, skin, lung, stomach and urine infections. If your child develops a very high temperature (above 38°C) or flu like symptoms you should contact the medical team for advice
- Effect on blood counts. This means certain components of your child's blood might be low, including platelets, haemoglobin and white blood cells. If you notice any unusual or unexplained bleeding or bruising you should contact your medical team for advice

- Aching joints
- Swelling around ankles and face (called oedema)
- Some patients develop a skin rash or acne
- Slow healing of wounds. This can be because of increased blood sugar levels so it is important to know the signs of high blood sugar levels including excessive thirst and increase in urination.

### Other side-effects and information

A small number of people may develop lung problems. This is rare but can sometimes be serious. If your child develops a cough or you notice that they are short of breath then please contact your medical team immediately for advice.

You should not wait until your child's next clinic visit as they may need to stop the tablets until your consultant has seen you.

Sirolimus may increase your child's risk of developing a blood clot (thrombosis) it is important to tell your doctor immediately if they have symptoms such as pain, redness and swelling in their leg, or breathlessness and chest pain.

Sometimes sirolimus can have very serious side-effects which rarely can be life threatening. It is important to inform your medical team if you are concerned about any side-effects.

If your child is due to have or is having an operation or any dental treatment, tell the person carrying out the treatment that your child is taking an immunosuppressive medicine

## Is it alright to take other medicines?

Sirolimus can react with some other medicines. If your child is taking or is started on any other medicines including over the counter or herbal medicines please tell your doctor, nurse or pharmacist.

### *The following medicines are known to react with sirolimus:*

- Other immunosuppressant medicines
- Certain antibiotics or antifungal medicines
- Anti-hypertension medicines
- Anti-epilepsy medicines
- Certain anti-ulcer medicines

Whilst your child is taking sirolimus they cannot receive any 'live' vaccinations due to their weaker immune system.

These include as MMR, oral polio, oral typhoid, rotavirus, chicken pox, nasal flu vaccine or BCG while taking sirolimus.

Inactivated or killed vaccines such as the influenza/flu (injection ONLY), meningitis C, pneumococcal, hepatitis, Hib, tetanus, diphtheria, whooping cough/pertussis and the killed version of the polio immunisation are permitted.

If you have other children who need vaccines while your child is taking sirolimus, they should have these as normal, but they should also receive the 'killed' or inactivated vaccine.

Please speak to a member of your medical team before having any vaccinations.

Your child should not have **grapefruit or grapefruit juice**. This is because a chemical in grapefruit juice increases the amount of sirolimus in your bloodstream. This makes side-effects more likely.

Due to an increased risk of skin cancer, exposure to sunlight and UV light should be limited by covering the skin with clothing and using sunscreen with a high protection factor.

If your doctor decides to stop treatment with sirolimus, please return any unused oral solution to the pharmacist. Do not flush it down the toilet or throw it away.

## **Manufacturer's patient information leaflets**

This patient information leaflet provide information about very common and commonly reported side-effects (we are unable to list all of the common side-effects), for more information regarding these and the less common side-effects please refer to the manufacturers patient information leaflets on the internet at **[www.medicines.org.uk](http://www.medicines.org.uk)**.

## **How do I arrange a repeat prescription of sirolimus?**

Sirolimus can only be supplied through the Hospital Outpatient Pharmacy on a hospital outpatient prescription. Sadly, we cannot arrange home delivery.

To ensure you don't run out, make sure you have at least a week's sirolimus left and contact us on the numbers below to arrange a repeat prescription.

## Contact telephone numbers

***In hours (9am - 5pm):*** CNS Interventional Radiology and Vascular Anomalies - **(0113) 392 0930** or **(0113) 392 0931**

***Out of hours:*** GP and or A&E stating your child is taking sirolimus and is at risk of febrile neutropenia.

***For example you should phone if your child is:***

- Sick more than once in 24 hours.
- Has a temperature of 38°C or above.
- Has four or more bowel movements in 24 hours above what is normal for you.
- Has a very sore mouth.
- Has a cough or shortness of breath.

For any queries about your medicines, please contact the patient medicines helpline Monday to Friday 9am to 5pm - **(0113) 206 4376**





## What did you think of your care?

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