



# Sirolimus

## ■ What is sirolimus and how does it work?

Sirolimus is an oral medication that blocks the mTOR pathway which regulates many important functions in the human body.

It was initially used to prevent rejection in solid organ (kidney) transplantation.

Besides its use in transplant patients, sirolimus is also used in the treatment of other conditions such as certain cancers, tuberous sclerosis, PTEN hamartoma syndromes and vascular anomalies.

## ■ What vascular anomalies have sirolimus been used to treat?

- Kaposiform haemangioendothelioma (KHE) and tufted angioma (TA) particularly with concurrent Kasabach-Merritt syndrome (KMS)
- Venous, lymphatic and veno-lymphatic malformations
- Blue rubber bleb nevus syndrome
- Some vascular anomalies syndromes e.g. Klippel-Trenaunay syndrome (KTS)
- Arteriovenous malformations (lower success rate)

## ■ What dose of sirolimus is used to treat vascular anomalies?

Sirolimus is usually started at a dose of 0.8 mg / m<sup>2</sup> / dose twice daily (BD) and adjusted according to drug levels in the blood.

Sometimes sirolimus is started at a lower dose if there are concerns of side effects. The doctor will inform you of the actual starting dose.

### What screening and monitoring blood tests are required before starting and while on sirolimus?

- You/your child will be required to undergo blood tests before and during treatment with sirolimus according to the doctor's instructions.
- Blood tests include full blood count, kidney panel, liver function test, serum cholesterol and triglycerides
- Urine test for urinary protein
- Blood pressure
- Sirolimus blood trough levels is done one hour before the next dose. This is checked 2 to 4 weeks after starting sirolimus or after the dose is increased. If stable, these may be checked 1 to 3 monthly depending on the doctor's advice.
- The target level of drug in the blood is 5 to 15 ng / ml.

### What are the side effects of sirolimus?

The most common side effect of sirolimus is mouth ulcers. This is more common in adolescents and adults compared to infants and children. This side effect is minimised by starting sirolimus at a lower dose. If this occurs, do inform your doctor. If it is severe, stop the medication and contact your doctor.

Other side effects include headache, insomnia, high blood pressure, leg swelling, skin rash and pimples, high cholesterol levels, diarrhoea or constipation, nausea and joint pains (arthralgia).

Abnormal blood tests include anaemia (low red blood cells), low platelets and low white blood cells, abnormal liver function tests, abnormal kidney panel.

### Are there any other drugs that i should avoid while on sirolimus?

Sirolimus can interact with other medications including some antibiotics (e.g. clarithromycin, rifampicin) and anti-fungals (e.g. itraconazole).

Please inform your child's other doctors that you/your child is on sirolimus. If there are any queries on the use of sirolimus and other medications, your doctor should contact a pharmacist for recommendations.

Dose adjustments may be required.

As sirolimus reduces the immune system of the body, vaccinations, especially with live vaccines should be avoided. Please inform the doctors that you/your child is on sirolimus before administration of any vaccines.

In young infants or patients at higher risk of infections, prophylactic antibiotics (eg. oral Bactrim twice a day on 2 days of the week) may be started together with sirolimus to reduce the risk of infections.

### Useful telephone number

Central Appointments

6294-4050



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