Information for the treatment of Immune Thrombocytopenia in Adults

ITP Australia New Zealand

Sirolimus

Third-line treatment

Sirolimus is an immunosuppressive medicine originally developed to prevent organ rejection after transplants. In ITP, it is considered a third-line treatment and is used off-label when first-line and second-line therapies haven't worked or aren't suitable.

It is often used in combination with other ITP treatments to improve platelet counts.

Sirolimus works by calming the immune system, which, in ITP, mistakenly attacks platelets. It blocks a pathway called mTOR, which helps certain immune cells grow and become active. By slowing down these cells, Sirolimus helps reduce platelet destruction and allows platelet levels to rise.

BRAND NAMES

Rapamune® - Also known as Rapamycin

HOW DOES THE TREATMENT WORK?

Sirolimus blocks a pathway in the immune system called mTOR (mammalian target of rapamycin). This pathway helps specific immune cells grow and become active.

By slowing this down, Sirolimus reduces the number of immune cells that destroy platelets.

HOW IS IT ADMINISTERED?

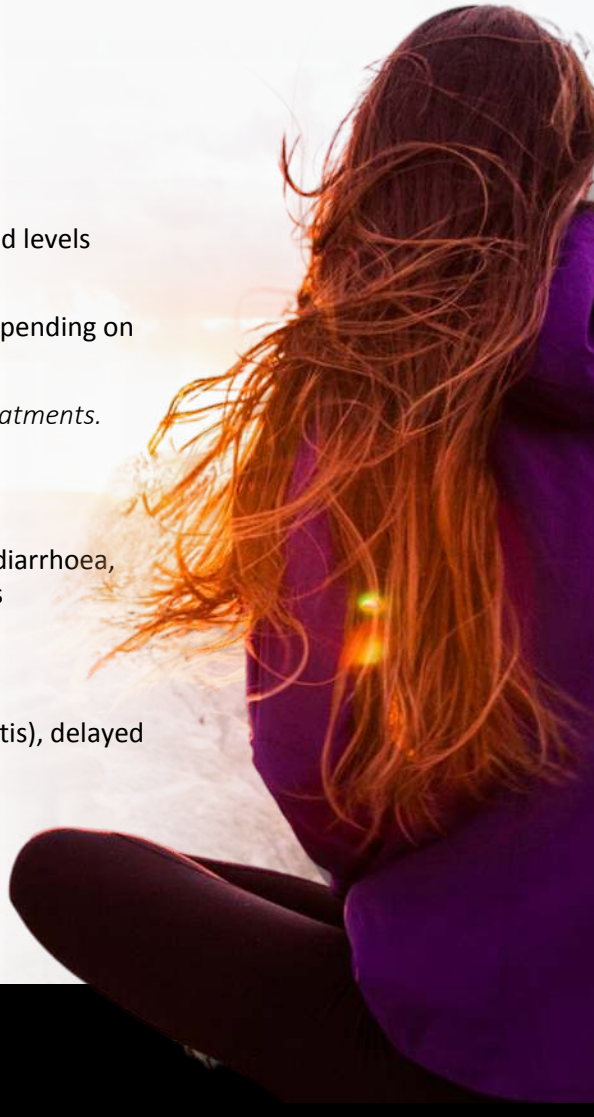
Taken orally as a tablet or liquid, usually once a day.

DOSAGE

The dose is based on body weight and adjusted depending on blood levels and response.

A common starting dose is around 2 mg daily, but this may vary depending on individual needs and monitoring results.

Note: This treatment is often used in combination with other ITP treatments.



COMMON SIDE EFFECTS

These can include mouth ulcers, high cholesterol or triglycerides, diarrhoea, headaches, acne or skin rash and/or an increased risk of infections

RARE SIDE EFFECTS

These can include kidney problems, Lung inflammation (pneumonitis), delayed wound healing and/or low blood counts (other than platelets)

TREATMENT RESPONSE

The median time to response is 4 to 8 weeks, though some people may take longer to respond.

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LIKELIHOOD OF AN INITIAL RESPONSE

Studies suggest that around 50 - 60% of adults with ITP may respond to Sirolimus initially.

LIKELIHOOD OF A LONG-TERM RESPONSE? *3-5 years*

Approximately 30 - 40% of patients may maintain a long-term response, especially if they respond well initially.

OTHER CONSIDERATIONS

With the treatment of Immune Thrombocytopenia, Sirolimus is generally used in combination with other first-line or second-line treatments. Please consult with the ITP Specialist or ITPANZ Medical Advisor for more information.

Regular blood tests are needed to monitor kidney function, cholesterol, and sirolimus levels.

Avoid grapefruit and grapefruit juice, as they can affect how the medicine works.

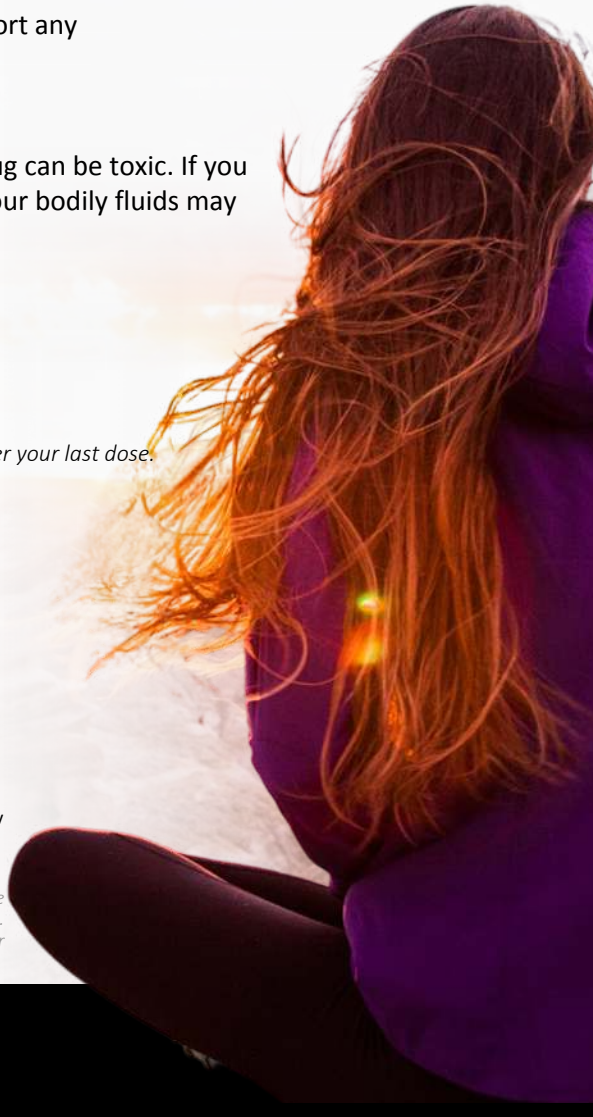
Sirolimus can increase the risk of infections, so it's essential to report any signs of illness early.

Not recommended during pregnancy or breastfeeding.

When this treatment is not used carefully and as intended, this drug can be toxic. If you become sick while taking this medicine *(for example, vomiting or diarrhoea)*, your bodily fluids may contain small amounts of the drug. To keep others safe:

- Wear gloves when cleaning up fluids or handling dirty laundry
- Wash your hands well after contact
- Wash contaminated clothes separately
- Avoid contact with bodily fluids if pregnant or breastfeeding
- Flush the toilet twice and clean the area if needed

Note: These precautions are most important during illness and for up to 7 days after your last dose.



REFERENCES

1. 2024 ANZ Consensus Guidelines for the Management of Adult Immune Thrombocytopenia (ITP)
2. PDSA Sirolimus Information Sheet (Adults) – Platelet Disorder Support Association
3. Provan D et al. "International consensus report on the investigation and management of primary immune thrombocytopenia." Blood 2010;115(2):168–186.
4. Cuker A et al. "American Society of Hematology 2019 guidelines for immune thrombocytopenia." Blood Adv 2019;3(23):3829–3866.