

5 year study of 6 children each year with vascular anomalies who have severe complications failing to respond to the usual treatments will be offered a new use of an old medication- sirolimus

ACTRN12616000943448

Status	RECRUITING
Phase	Phase 1
Sponsor	Sydney Children's Hospital, Randwick
Enrollment	30 participants

Plain Language Summary

This study is for children with serious and rare blood vessel abnormalities (called vascular anomalies) that have not responded to standard treatments. These conditions can cause pain, disfigurement, or life-threatening problems. This trial tests the safety and effectiveness of a medicine called sirolimus, which has shown promising results in similar cases around the world.

You may be eligible if:

- The child is between newborn and 18 years old
- The child has a complex vascular anomaly such as venous malformation, lymphatic malformation, Kaposiform haemangioendothelioma, or overgrowth syndromes
- The condition has not responded to surgery, sclerotherapy, or other standard treatments, or there is no accepted standard treatment available

You may NOT be eligible if:

- The child has severe immune system problems (severely immunocompromised)

Talk to your doctor about whether this trial might be right for you.

Key Eligibility Criteria

Inclusion (7)

- Venous Malformations with localised intravascular coagulopathy or severe pain failing to respond to sclerotherapy and /or surgery;
- Lymphatic Malformations which compromise the airway or vision or are causing a low albumin level;
- Newly diagnosed Kaposiform haemangioendothelioma/tufted angioma: in view of the promising data from the US on the use of sirolimus as initial treatment for KHE (1,2,5), sirolimus will be given to newly diagnosed patients and the results compared to our historical controls, namely children who had received vincristine as initial therapy.
- Relapsed Kaposiform haemangioendothelioma: children who have previously been successfully treated with vincristine therapy and then have a recurrence of their tumour will be treated with sirolimus;
- Generalised Lymphatic Anomalies with bone erosions, chylous leak or infiltration of lungs and gastrointestinal tract;
- ... and 2 more (see full listing online)

Exclusion (1)

- Children who are severely immunocompromised will be excluded from the study.

Locations (1 total)

Sydney Children's Hospital - Randwick, NSW, Australia

<https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?ACTRN=ACTRN12616000943448>

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