

Pentosan Polysulfate Sodium (PPS) in subjects with mucopolysaccharidosis type I (MPS I)

ACTRN12620000823976

Status	RECRUITING
Phase	Phase 2
Sponsor	Paradigm Biopharmaceuticals Pty Ltd
Enrollment	10 participants

Plain Language Summary

Mucopolysaccharidosis Type I (MPS I) is a rare inherited condition where the body cannot properly break down certain complex sugars, which then build up in cells and tissues and cause progressive damage. It affects multiple systems including the skeleton, joints, heart, and nervous system. People with MPS I commonly experience significant pain and functional limitations even after receiving standard treatments like enzyme replacement therapy (ERT) or bone marrow transplantation.

This study is testing whether a drug called Pentosan Polysulfate Sodium (PPS) — already used for other conditions — can safely reduce pain and improve function in MPS I patients. Participants will receive PPS as a weekly injection (then fortnightly), and the study will run for about 73 weeks. The study is open-label, meaning everyone will know what treatment they are receiving, and up to 10 participants aged 5 and over will be enrolled.

You may be eligible if you are aged 5 or older, have a confirmed MPS I diagnosis, have already received standard treatment (bone marrow transplant and/or ERT), and are able to walk independently (with or without a walking aid). People with bleeding disorders, on blood-thinning medications, or with a history of stomach ulcers would not be eligible.

Key Eligibility Criteria

Inclusion (6)

- Males and females aged greater than or equal to 5 years
- Documented diagnosis of MPS I
- Must have received primary therapy for MPS I (HSCT (Haemopoietic stem cell transplantation) +/- ERT (enzyme replacement therapy))
- For subjects receiving ERT, ERT must have been administered at a stable dose
- Able to walk independently with or without use of an assistive device

... and 1 more (see full listing online)

Exclusion (16)

- Documented or reported history of increased bleeding tendency in the presence or absence of anticoagulant or antiplatelet drugs
- History of idiopathic or immune-mediated (including heparin-induced) thrombocytopenia
- Current treatment with anticoagulants or antiplatelet drugs, excluding aspirin less than or equal to 100 mg/day
- Use of opioids or medications on the Prohibited Medications list within 2 weeks of Day 1 and throughout the duration of the study
- Currently active or recent history (within preceding 12 months) of a gastric or duodenal ulcer, or suspicion of GI tract bleeding

... and 11 more (see full listing online)

Locations (1 total)

SA, Australia
<https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?ACTRN=ACTRN12620000823976>

DISCLAIMER: This document is for informational purposes only and does not constitute medical advice. Always consult your healthcare provider before enrolling in any clinical trial. Information may not be up to date — verify details at anzctr.org.au. Generated by ClinicalTrialsFinder.org.