Phase I Trial of Deflexifol for Refractory or Recurrent Paediatric Central Nervous System (CNS) Tumours

ACTRN12623000104651

Status	RECRUITING
Phase	Phase 1
Sponsor	Australian and New Zealand Children's Haematology and Oncology Group (ANZCHOG)
Enrollment	24 participants

Plain Language Summary

Brain tumours in children and young people are among the most difficult cancers to treat. When a tumour comes back (recurs) or stops responding to treatment (becomes refractory), options are extremely limited. Deflexifol is a new formulation of the chemotherapy drug 5-fluorouracil that has been designed to work differently from existing treatments and may be active against brain tumours in this setting.

This Phase I study is the first step in testing Deflexifol specifically in children, adolescents, and young adults. The primary goal is to establish that it is safe and to find the right dose. Participants will receive Deflexifol every two weeks via an intravenous infusion — a short bolus injection followed by a 46-hour continuous infusion — for up to approximately one year if the treatment appears to be helping. Careful monitoring will include blood tests, MRI scans, heart tests, and quality of life assessments throughout.

You may be eligible if you are older than 12 months and up to 21 years of age and have a recurrent or treatment-resistant brain tumour, or if you have been newly diagnosed with diffuse intrinsic pontine glioma (DIPG) or diffuse midline glioma (DMG) and have completed radiotherapy. This is a multicentre study for a patient population where new treatments are urgently needed.

Key Eligibility Criteria

Inclusion (18)

- Written informed consent
- Patients must be greater than 12 months and less than or equal to 21 years of age at the time of study enrolment
- Patient must have histologic confirmation of CNS tumour that is refractory or recurrent based on radiologic or tissue diagnosis; and for which there is no known curative therapy or therapy proven to prolong survival with an acceptable quality of life.
- Patient must have measurable disease as per RAPNO criteria and/or evaluable disease. For patients who have received prior radiation therapy, the irradiated lesion needs to have progressed since cessation of radiotherapy.
- Karnofsky performance status greater than or equal to 50 for patients above 16 years old;; or Lansky performance status greater than or equal to 50 for patients less than or equal to 16 years old. Patients who are not able to walk due to paralysis, but who are sitting up in a wheelchair, are considered ambulatory for the purpose of performance score assessment

... and 13 more (see full listing online)

Exclusion (7)

- Concomitant medications
- a. Patients who are currently receiving another investigational agent are not eligible
- b. Patients who are currently receiving other anti-cancer agents are not eligible
- Any known Dihydropyrimidine dehydrogenase (DPD) deficiency
- Prior solid organ transplantation

... and 2 more (see full listing online)

Locations (8 total)

https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?ACTRN=ACTRN12623000104651

Sydney Children's Hospital - Randwick, NSW,QLD,SA,WA,VIC, Australia
The Children's Hospital at Westmead - Westmead, NSW,QLD,SA,WA,VIC, Australia
John Hunter Children's Hospital - New Lambton, NSW,QLD,SA,WA,VIC, Australia
... and 5 more locations