

HREC/19/QCHQ/58164

A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Phase 3 Study to Evaluate the Efficacy and Safety of OV101 in Pediatric Individuals With Angelman Syndrome (NEPTUNE)

Children’s Health Queensland is currently conducting a clinical trial investigating the safety and efficacy of OV101 (Gaboxadol) for the treatment of symptoms related to Angelman Syndrome i.e. sleep, communication, socialisation, seizures.

We are seeking male and female participants aged 2- 12 years with a confirmed diagnosis of Angelman Syndrome (molecular confirmation) to undertake 12 weeks of double-blind treatment (50% receiving drug; 50% receiving placebo). Treatment involves taking the drug in capsule form (capsules can be opened and sprinkled onto soft food) each evening for the duration of the 12-week treatment period. At the end of the double-blind treatment period, participants will have the option to enrol in the ELARA study, which is the open-label (each participant receives the study drug) extension study.

As this is a safety and efficacy study, participants will be required to have blood tests, ECGs and vital signs taken throughout the study, so that the study team may effectively monitor their safety whilst taking the study drug.

Study visits will take place at the Centre for Children’s Health Research Clinical Research Facility, located behind the Queensland Children’s Hospital at South Brisbane.

If you are interested in this study and would like further information, please contact the Centre for Clinical Trials in Rare Neurodevelopmental Disorders study team via 07 3069 7532 or CHQ_CCTRND@health.qld.gov.au

