



GTX-102 for Angelman syndrome Feasibility Questionnaire

Dr. Aneal Khan at MAGIC Clinic Ltd. in Calgary is considering a study in patients with Angelman syndrome using an investigational (research) drug called GTX-102.

The study has not yet been approved in Calgary and right now we are trying to determine how many patients may be interested and whether Calgary should be a site for the study. Dr. Khan is conducting a feasibility questionnaire to see how many patients may be eligible - the information is anonymous and will not identify anyone. We simply want a rough idea of eligible patients. Formal determination of eligibility will only take place after ethics approval is complete and we can proceed with enrolment. Answering the questionnaire does not place you on a waiting list for the study nor put you in cue to be contacted if the study starts. We do not collect any personal identifying information or contact information.

You will find other sites for this study listed at the following website:

<https://www.clinicaltrials.gov/ct2/show/NCT04259281?term=gtx-102&draw=2&rank=1>

Things you need to know:

- answering the questionnaire is voluntary
- We do not record or store who answered the questionnaire and do not add people to the waiting list for the study or on any enrolment list
- The study is not yet approved in Calgary at Dr. Khan's site
- Patients must be between 4 - 17 years of age at the time of inclusion in the study
- Only patients with a diagnosis of Angelman syndrome caused by a *UBE3A* gene deletion (within the 15q11.2-q13) with a confirmed genetic test are eligible

What is the objective of the study ?

The objective of the study is to look at the safety and tolerability of the drug GTX-102 which is given as an injection into the spinal fluid using a needle (called an intrathecal injection). Some children may need anesthesia in order to give the injection. This is a Phase 1/2 study with different doses of GTX-102 and no placebo.

What does the study require that patients go through ?

Patients will have a number of tests performed and then go through a spinal injection of GTX-102 at baseline and then 2 to 3 more doses approximately 28 days, 56 days and/or 84 days later. After this, the patients will receive a spinal injection of GTX-102 every 3 months. After each injection, patients will have to lie relatively still for 45 minutes for the drug to move up from the spine to the brain from gravity. Patients will lie with the head tilted down a few degrees to help with movement of the drug. Overall, the study will last about 6 months from start to finish. Patients will have a physical exam, neurological exam, measurement of vital signs, blood and urine tests, a heart rhythm tracing (electrocardiogram or ECG), analysis of spinal fluid, keep a diary of symptoms and seizure control, and have assessments of motor function and developmental skills. A brain MRI is optional.

Where will the study be conducted ?

Canadian patients who enrol through Dr. Aneal Khan will be treated at the MAGIC Clinic in Calgary, Alberta, Canada (<https://magiccalgary.ca>). For families that need to bring a child to Calgary for the study, the study is expected to provide some recovery of travel costs.

Where can I enter responses to the anonymized survey ?

Simply scan this QR code through the camera on your phone



Or go to the website:

<https://www.surveymonkey.com/r/PTZST26>

Thank you for your interest,
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