Phase 1/2 Clinical Trial of TSHA-101 for Infantile GM2 Gangliosidosis: Information for Families

Who is conducting this trial?

The clinical trial is sponsored and conducted by Queen's University in Ontario, Canada. The trial received financial support from the Canadian Glycomics Network (GlycoNet) and Taysha Gene Therapies, Inc., a patient-centric gene therapy company focused on monogenic diseases of the central nervous system.

Where will this clinical trial take place?

The trial will be conducted at Kingston Health Sciences Center at Queen's University in Kingston, Ontario, Canada.

Will this trial enroll children who live outside of Canada?

Yes, this is possible. The trial will enroll children wherever they and their caregivers reside. Clinical trial visits will be conducted in person at Kingston Health Sciences Center at Queen's University in Kingston, Ontario, Canada. Non-Canadian families will be required to obtain travel health insurance coverage for the duration of their stay in Canada.

Who can participate in the TSHA-101 clinical trial?

Taysha's top priority is finding a safe and effective gene therapy treatment for children with Tay-Sachs disease or Sandhoff disease. Doing so involves conducting a clinical trial with children who meet very specific criteria. Though not every child with Tay-Sachs disease or Sandhoff disease will qualify, the research may show how this investigational gene therapy could help children who have these diseases.

With that in mind, the clinical trial has been carefully designed to maximize our understanding of the investigational gene therapy. Children aged less than 12 months with a confirmed diagnosis of Tay-Sachs disease or Sandhoff disease may be screened to participate in this trial. In addition, the parent or caregiver and participating child will need to reside within 30 kilometers (approximately 19 miles) of the research site during the 1-month screening period and until at least 3 months after gene transfer, for a total of 4 months.

There are some criteria that would make a child unable to participate in the trial. We encourage any family considering this trial to contact the research team to discuss these criteria in more detail, and to learn more about whether their child would be eligible to participate.

Why is TSHA-101 only being studied in infantile GM2 gangliosidosis (children less than 12 months of age)?

Patients with the infantile form of Tay-Sachs disease or Sandhoff disease are the most vulnerable population and need the treatment most urgently.

The ideal time to start therapy for diseases that affect brain cells is before significant, irreversible injury develops. For this reason, much of the attention of early clinical development programs has focused on younger children. Taysha recognizes the critical need of the whole population with GM2 gangliosidosis, particularly older children with all forms of the disease. Taysha is evaluating the steps necessary to study therapies for older patients.

Are there plans to study TSHA-101 in older children?

Taysha recognizes the critical need of the whole population with GM2 gangliosidosis, particularly older children with all forms of the disease. Taysha is evaluating the steps necessary to study therapies for older patients. Data generated from this trial will help inform future research plans, including which populations could benefit from TSHA-101.

What will it cost to participate in this clinical trial?

TSHA-101 is an investigational gene therapy that will be supplied at no charge to families while their child participates in the trial. **Parents/guardians of a study participant will receive reimbursement for eligible and appropriate expenses incurred while in Kingston, Ontario, Canada for clinical trial site visits:**

- Driving/mileage
- Tolls/parking
- Flights
- Accommodations
- Rental cars
- Public transportation/taxi
- Meals/food
- Travel medical insurance

Who should a family contact if they have additional questions?

Families interested in learning more about this clinical trial and if it would be a good option for their child can email the Queen's University research team at info@TSD-SDGTXtrial.com.