

December 21, 2020

## Dear Tay-Sachs and Sandhoff Community,

We are pleased to share news that Queen's University in Ontario, Canada, has received Clinical Trial Application (CTA) approval from Health Canada for the Phase 1/2 clinical trial of TSHA-101, Taysha's investigational gene therapy for the treatment of infantile GM2 gangliosidosis. CTA approval means that the regulatory authorities in Canada have reviewed and approved the clinical trial plans for TSHA-101.

TSHA-101 is designed to deliver functional copies of HEXA and HEXB genes in equal quantities through a single AAV9 vector. This is called a bicistronic transgene. Equal delivery is important for building the functional protein, which consists of one piece each of two different protein units.

Approval of the TSHA-101 clinical trial plans by Health Canada is an important milestone, and we are encouraged by the progress that Queen's University is making toward initiating this clinical trial.

*"Today's CTA approval is a culmination of our team's and Dr. Walia's tireless efforts and a momentous occasion for children affected by GM2 along with their parents and caregivers. We are grateful to our partners at Queen's University for their work to advance this gene therapy into the clinic."*

*RA Session II, Founder, President and CEO  
Taysha Gene Therapies*

Taysha is grateful for the support of the GM2 gangliosidosis communities. When new information is available, we looking forward to providing updates about the TSHA-101 clinical trial through the global patient organizations.

Warm regards,

**The Taysha Team**