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.

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