Taysha Gene Therapies Announces Queen's University's Receipt of Clinical Trial Application Approval from Health Canada for Phase 1/2 Clinical Trial of TSHA-101 for the Treatment of Infantile GM2 Gangliosidosis

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TSHA-101 to be first bicistronic vector evaluated in human clinical trials; TSHA-101 designed to deliver both HEXA and HEXB transgenes within a single AAV9 vector construct

TSHA-101 CTA is the second clinical trial clearance received, in addition to TSHA-118's open investigational new drug application for CLN1

Interim data from Phase 1/2 trial anticipated in 2021

DALLAS--(BUSINESS WIRE)--Dec. 21, 2020-- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations, today announced that Queen's University in Ontario, Canada, received Clinical Trial Application (CTA) approval from Health Canada for its investigator-sponsored Phase 1/2 trial exploring TSHA-101, Taysha's investigational AAV9-based gene therapy, for the treatment of infantile GM2 gangliosidosis.

"TSHA-101 will be the first bicistronic vector to enter a first-in-human clinical study, which is a significant milestone for Taysha and for the field of gene therapy," said Suyash Prasad, MBBS, M.SC., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. "GM2 is a devastating lysosomal storage disease with no approved treatments and today's CTA approval marks a formative moment for children suffering from this rapidly progressive and fatal disease."

The trial will be a single arm, open-label Phase 1/2 trial evaluating the use of TSHA-101 for the treatment of infants with GM2. The study will be sponsored by Queen's University and led by Jagdeep S. Walia, MBBS, FRCPC, FCCMG, Clinical Geneticist and Associate Professor Head, Division of Medical Genetics (Department of Pediatrics) at Queen's, and Director of Research (Department of Pediatrics), at the Kingston Health Sciences Centre.

"Preclinical evidence to date supports our belief that TSHA-101, when given intrathecally as a bicistronic transgene packaged into a single AAV9 vector, has the potential to address the lysosomal enzyme deficiency, to change the disease trajectory and to improve patient survival," said Dr. Jagdeep S. Walia. "We are pleased to have the support of Health Canada as we continue to advance TSHA-101."

"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," said RA Session II, Founder, President and CEO of Taysha. "We are grateful to our partners at Queen's University for their work to advance this gene therapy into the clinic."

About GM2 Gangliosidosis

GM2 gangliosidosis is a rare and fatal monogenic lysosomal storage disorder and a family of neurodegenerative genetic diseases that includes Tay-Sachs and Sandhoff diseases. The disease is caused by defects in the *HEX*A or *HEX*B genes that encode the two subunits of the β-hexosaminidase A enzyme. These genetic defects result in progressive dysfunction of the central nervous system. There are no approved therapies for the treatment of the disease, and current treatment is limited to supportive care.

About TSHA-101

TSHA-101 is an investigational gene therapy administered intrathecally for the treatment of infantile GM2 gangliosidosis. The gene therapy is designed to deliver two genes – HEXA and HEXB – driven by a single promoter within the same AAV9 construct, also known as a bicistronic vector. This approach allows the simultaneous expression of a 1:1 ratio of the two subunits of protein required to generate a functional enzyme. It is the first and only bicistronic vector currently in clinical development and has been granted Orphan Drug and Rare Pediatric Disease designations by the U.S. Food and Drug Administration (FDA).

About Taysha Gene Therapies

Taysha Gene Therapies (Nasdaq: TSHA) is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goa of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as

“anticipates,” “believes,” “expects,” “intends,” “projects,” and “future” or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning or implying the potential of our product candidates, including TSHA-101, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed. Forward-looking statements are based on management’s current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission (“SEC”) filings, including in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, which is available on the SEC’s website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

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