Taysha Gene Therapies Announces Collaboration with AllStripes on SURF1-Associated Leigh Syndrome Clinical Development and Natural History

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Taysha to leverage AllStripes' technology platform to support development of TSHA-104 for SURF1-associated Leigh syndrome

DALLAS--(BUSINESS WIRE)--Jan. 4, 2021-- 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 a multi-year collaboration with AllStripes (formerly RDMD), a healthcare technology company dedicated to accelerating research for patients with rare diseases, during which Taysha will leverage AllStripes' platform to inform its understanding of SURF1-associated Leigh syndrome natural history and burden of disease, as well as patients' diagnostic journeys.

The collaboration will focus on advancing the development of TSHA-104, an AAV9-based gene therapy product candidate in development for the treatment of SURF1-associated Leigh syndrome. Taysha will utilize AllStripes' clinical database to uncover new insights into disease progression and better inform selection of endpoints for clinical studies.

"This collaboration will allow us to leverage the AllStripes technology platform to optimize our therapeutic strategy and to potentially accelerate the development of TSHA-104 in SURF1-associated Leigh syndrome," said RA Session, II, President, Founder and Chief Executive Officer of Taysha. "We remain committed to developing a safe and effective gene therapy for patients suffering with this devastating disease, and data generated from this unique collaboration could bring us one step closer to our goal."

"Taysha has brought together accomplished and knowledgeable gene therapy and CNS disease experts to develop potentially transformative therapies," said Nancy Yu, Co-founder and Chief Executive Officer of AllStripes. "With no available treatment for SURF1-associated Leigh syndrome, we are very pleased to empower patients and their families with an avenue to participate in research that will support the development path of TSHA-104. We are hopeful that this novel gene therapy will bring meaningful benefit to children and their families, and give them more time together."

TSHA-104 has been granted rare pediatric disease and orphan drug designations from the U.S. Food and Drug Administration (FDA) for the treatment of SURF1-associated Leigh syndrome. An Investigational New Drug (IND) application for TSHA-104 in SURF1-associated Leigh syndrome is expected to be submitted to the FDA in 2021.

About SURF1-Associated Leigh Syndrome

SURF1 deficiency is a monogenic mitochondrial disorder and is the most common cause of cytochrome c oxidase deficient Leigh syndrome. Leigh syndrome is a severe neurological disorder that usually presents in the first year of life. It is characterized by progressive loss of mental and movement abilities that can result in death within two to three years. Approximately 10-15% of people with Leigh syndrome have a SURF1 mutation. There are currently no approved therapies to treat SURF1-associated Leigh syndrome.

About TSHA-104

TSHA-104 is an investigational AAV9-based gene therapy administered intrathecally for the treatment of SURF1-associated Leigh syndrome. The product candidate is a recombinant AAV9 vector with engineered transgene encoding the human SURF1 protein. TSHA-104 has been granted Orphan Drug and Rare Pediatric Disease designations by the 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.

About AllStripes

AllStripes is a healthcare technology company dedicated to unlocking new treatments for people with rare diseases. AllStripes has developed a technology platform that generates FDA-ready evidence to accelerate rare disease research and drug development, as well as a patient application that empowers patients and families to securely participate in treatment research online and benefit from their own medical data. AllStripes was founded by CEO Nancy Yu and technology developer Onno Faber, following his diagnosis and journey with the rare disease neurofibromatosis type 2. The company is backed by Lux Capital, Spark Capital, Maveron Capital, Village Global, Garuda Ventures and a number of angel investors. For more information, visit www.allstripes.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 collaboration with AllStripes, the potential of our product candidates, including TSHA-104, 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 benefits of rare pediatric disease designation and orphan drug designation to 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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