Taysha Gene Therapies Announces Sponsored Genetic Testing for Giant Axonal Neuropathy (GAN) in Partnership with GeneDx as well as a Collaboration with Hereditary Neuropathy Foundation and Charcot-Marie-Tooth Association Centers of Excellence

GeneDx's Hereditary Neuropathy Panel will be available free of charge to individuals at risk for or suspected of having GAN

Taysha will collaborate with Hereditary Neuropathy Foundation and Charcot-Marie-Tooth Association Centers of Excellence to increase disease awareness and access to testing

TSHA-120 is the first and only gene therapy to demonstrate clinical-stage arrest of disease progression at the $1.2x10^{14}$ and $1.8x10^{14}$ total vg dose

Clinical data for high dose $3.5x10^{14}$ total vg cohort expected in the second half of 2021 with regulatory feedback by year-end 2021

Estimated prevalence of patients with GAN is 2,400 in United States and Europe

DALLAS--(BUSINESS WIRE)--Oct. 13, 2021-- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced sponsored genetic testing for giant axonal neuropathy (GAN) in partnership with GeneDx, Inc., a leader in genomic analysis and a wholly owned subsidiary of BioReference Laboratories, Inc., an OPKO Health company (NASDAQ:OPK). Under the partnership, Taysha will sponsor the inclusion of a genetic marker to test for GAN in the GeneDx hereditary neuropathy panel free of charge to individuals at risk for or suspected of having GAN.

GAN is a progressive neurodegenerative disease that affects both the central and peripheral nervous systems leading to motor weakness, sensory impairment, and cognitive dysfunction. Currently, there are no approved treatments for GAN, which results in death for patients often in their late teens or early twenties. Although no symptoms are present in the first few months of life, many children with early onset GAN develop symptoms and features before the age of three. A more recently identified later onset phenotype of GAN is often mischaracterized as Charcot-Marie-Tooth. The estimated prevalence for GAN is 2,400 patients, but the GAN population is anticipated to be larger than previously appreciated.

In the natural history study, Motor Function Measure 32 (MFM32), a validated and well-known scale to measure strength and motor function, is the primary endpoint. A four-point change on the MFM32 score is considered clinically meaningful, which has been validated across numerous similar neuropathies. Natural history decline in patients with GAN is eight points per year. Treatment with TSHA-120, Taysha's intrathecally dosed AAV9 gene therapy currently being evaluated in a Phase 1/2 clinical trial led by Carsten Bönnemann, M.D., Chief of the Neuromuscular and Neurogenetic Disorders of Childhood Section and Senior Investigator, Neurogenetics Branch of the National Institute of Neurological Disorders and Stroke (NINDS), U.S. National Institutes of Health (NIH). Data demonstrate a statistically significant halt in decline as measured by the MFM32 at therapeutic doses. All GAN natural history data was generated and supported by the NINDS, NIH under umbrella protocol NCT01568658.

"Rare diseases like GAN are often mischaracterized, particularly when there are overlapping symptoms with other diseases with higher awareness," said Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. "Early diagnosis can dramatically improve the lives of patients and we are very excited to work with GeneDx, a global leader in genetic testing, to have GAN included in its routine hereditary neuropathy screening panel. Ultimately, this can help address current treatment barriers by raising disease awareness, making diagnostic tools more accessible and facilitating earlier intervention for patients suffering from GAN. We are also excited to collaborate with the Hereditary Neuropathy Foundation and the Charcot-Marie-Tooth Association Centers of Excellence, healthcare professionals, and patient advocacy groups to increase access to genetic testing. In the meantime, we look forward to reporting Phase 1/2 clinical data for TSHA-120 from the high dose cohort in the second half of this year. We have submitted a request for scientific advice from a regulatory agency and look forward to next steps."

Paul Kruszka, M.D., F.A.C.M.G., chief medical officer of GeneDx added, "Earlier access to genetic testing can take years off the diagnostic journey for patients facing rare diseases like GAN. By ensuring patients and clinicians no longer have to rely on solely clinical criteria and symptom presentation to get to an accurate diagnosis, we can help clinicians turn their attention to effective treatment plans earlier in the course of the disease. We look forward to partnering with Taysha on this important effort to improve diagnosis of neurodegenerative diseases like GAN."

Taysha will sponsor the new initiative which will make genetic testing available free of charge to individuals at risk for or suspected of having GAN. The initiative is designed to increase access to genetic testing for patients. GeneDx will provide analysis of genes on the Hereditary Neuropathy Panel by next-generation sequencing with deletion/duplication detection.

As the later onset phenotype of GAN is sometimes miscategorized as Charcot-Marie-Tooth or Charcot-Marie-Tooth Type 2, Taysha plans to collaborate with the Hereditary Neuropathy Foundation (HNF) and the Charcot-Marie Tooth Association (CMTA) by engaging their Centers of Excellence to administer genetic tests to appropriate patients and by creating awareness among patients and families about this new initiative.

Allison Moore, Founder and Chief Executive Officer (CEO) of the HNF said, "We believe all patients at risk for or suspected to have GAN should have access to receive genetic testing in order to confirm a potential GAN diagnosis and to be made aware of opportunities to participate in clinical trials for investigational treatments. This initiative aligns with HNF's mission to accelerate and help facilitate an accurate diagnosis for patients and families worldwide."

"Through this initiative, patients will have access to no-charge genetic testing, eliminating one of the biggest barriers," said Amy Gray, CEO of the CMTA. "The CMTA's support of this initiative is part of our broader commitment to accelerate research and empower patients and families living with GAN and other forms of CMT, including the most vulnerable, by giving them access to resources that may help improve outcomes and quality of life."

All testing will be ordered through a clinician. Sponsored testing programs have previously been shown to increase access to genetic testing, confirm diagnosis and enable participation in clinical trials for patients.

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 goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

About GeneDx, Inc.

GeneDx, Inc. is a global leader in genomics, providing testing to patients and their families worldwide. Originally founded by scientists from the National Institutes of Health, GeneDx offers a world-renowned clinical genomics program with particular expertise in rare and ultra-rare genetic disorders. In addition to its market-leading exome sequencing service, GeneDx offers a suite of additional genetic testing services, including diagnostic testing for hereditary cancers, cardiac, mitochondrial, neurological disorders, prenatal diagnostics, and targeted variant testing. GeneDx is a subsidiary of BioReference Laboratories, Inc., a wholly owned subsidiary of OPKO Health, Inc. To learn more, please visit http://www.genedx.com.

About OPKO Health

OPKO Health, Inc. (NASDAQ:OPK) is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large, rapidly growing markets by leveraging its discovery, development, and commercialization expertise and novel and proprietary technologies. For more information, visit www.opko.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 the potential of our product candidates, including TSHA-120, 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, TSHA-120's eligibility for accelerated approval in the United States and Europe, 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, and the potential market opportunity for these product candidates. Forward-looking statements also include statements as to the accuracy of GeneDx testing and the impact of testing on treatment paths and outcomes. Forward-looking statements are based on 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 the respective Annual Report on Form 10-K for the full-year ended December 31, 2020, and our Quarterly Reports on Form 10-Q for us and OPKO, all of which are 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 and OPKO disclaim any obligation to update these statements except as may be required by law.

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