Taysha Gene Therapies Reports Full-Year 2020 Financial Results and Provides a Corporate Update

March 3, 2021

Expects preliminary Phase 1/2 safety and biomarker and preliminary clinical data for TSHA-101 in GM2 gangliosidosis from the Queen's University study in second half of 2021 and by year-end 2021, respectively

Plans to initiate Phase 1/2 clinical trial for TSHA-118 in CLN1 disease in the second half of 2021 and Phase 1/2 clinical trials for TSHA-102 in Rett syndrome and TSHA-104 in SURF1-associated Leigh syndrome by year-end

Plans to initiate a U.S. Phase 1/2 clinical trial for TSHA-101 in GM2 gangliosidosis in second half of 2021

Expects IND/CTA submission from one of the following programs: SLC13A5 deficiency, APBD, Lafora disease, GM2 AB variant and SLC6A1 haploinsufficiency

Conference call and webcast today at 8:00 AM Eastern Time

DALLAS--(BUSINESS WIRE)--Mar. 3, 2021-- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric, clinical-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 reported financial results for the full-year ended December 31, 2020 and provided a corporate update.

"In 2020, we successfully completed one of the fastest seed to IPOs in biotech history and made significant strides in advancing our pipeline initiatives, next-generation technology platforms and manufacturing strategy," said RA Session II, President, Founder and CEO of Taysha. "We expect 2021 to be a transformational year for Taysha, as we anticipate reporting first-in-human clinical data for TSHA-101 in GM2 gangliosidosis and initiating Phase 1/2 trials in CLN1 disease, Rett syndrome and SURF1-associated Leigh syndrome. We will be submitting multiple IND/CTAs across three CNS franchises, and advancing four gene therapies into IND-/CTA-enabling studies and initiating work on additional targets. We continue to enhance our platform of next-generation technologies to optimize key components of our approach with AAV-based gene therapies. With recent additions to our team and Board of Directors and the formation of our Scientific Advisory Board, we believe we have the resources needed to achieve our corporate initiatives this year. Lastly, we will continue to evaluate other opportunities to maximize the value of our pipeline and technology platforms. We look forward to providing updates on our progress throughout the year and at an R&D day later this year."

2020 Corporate Highlights and Updates

Since inception a year ago, the company has achieved its strategic priorities, as highlighted by:

- Health Canada's approval of Queen's University's clinical trial application (CTA) for the Phase 1/2 clinical trial of TSHA-101 for the treatment of GM2 gangliosidosis
- Expanded portfolio of CNS gene therapy programs across three distinct franchises from 18 to 25 product candidates
- A portfolio of rare pediatric disease and orphan drug designations obtained in multiple pipeline programs, including TSHA-101 for GM2 gangliosidosis, TSHA-102 for Rett syndrome, TSHA-103 for SLC6A1 haploinsufficiency disorder, TSHA-104 for SURF1-associated Leigh syndrome, TSHA-105 for epilepsy caused by SLC13A5 deficiency and TSHA-118 for CLN1 disease
- Continued progress on next-generation technology platform, including achievement of animal proof-of-concept for vagus nerve redosing
- Expanded partnership with UT Southwestern (UTSW) through launch of an innovation fund to accelerate advancement of AAV gene therapies for the treatment of monogenic diseases of the CNS
- Collaboration established with Cleveland Clinic and UTSW to advance next-generation mini-gene payloads for AAV gene therapies for the treatment of genetic epilepsies and additional CNS disorders
- Collaborations established with Invitae and AllStripes to support access to genetic testing and earlier diagnosis of patients with CNS diseases and to inform the understanding of the natural history and disease burden of and patients' diagnostic journeys with SURF1-associated Leigh syndrome, respectively
- Manufacturing partnerships established with UTSW and Catalent and entered into a lease agreement to occupy and configure an approximately 187,000-square-foot commercial-scale current Good Manufacturing Practices (cGMP) manufacturing facility in Durham, North Carolina to support preclinical, clinical and commercial production for Taysha's broad pipeline of gene therapies
- Appointment of industry-leading gene therapy executives, Kathy Reape, M.D., former Chief Medical Officer of Spark Therapeutics, and Laura Sepp-Lorenzino, Ph.D., Chief Scientific Officer of Intellia Therapeutics, to the Board of Directors

- Formation of independent Scientific Advisory Board consisting of preeminent international scientific and clinical thought leaders in gene therapy, CNS diseases and drug discovery and development
- Expansion of leadership team to add significant gene therapy expertise and to deepen manufacturing, human resources, legal and corporate communications capabilities
- The company has grown from a handful of employees at inception to approximately 80 as of the end of February
- Gross proceeds of approximately $307 million raised since the company's formation, including $181 million in gross proceeds from IPO

Full-Year 2020 Financial Highlights

Research and Development (R&D) Expenses: R&D expenses were $31.9 million for the year ended December 31, 2020 compared to $1.0 million for the period from September 20, 2019 (the date of company inception) to December 31, 2019. The increase was primarily related to the company's development programs, as a result of increased manufacturing-related spend, clinical and preclinical activities, and headcount.

General and Administrative (G&A) Expenses: G&A expenses were $11.1 million for the year ended December 31, 2020 compared to $0.1 million for the period from September 20, 2019 (the date of company inception) to December 31, 2019. The increase was primarily due to an increase in personnel costs resulting from increased headcount, professional services fees, and other corporate-related expenses.

Other Expenses: Other expenses were $17.0 million for the year ended December 31, 2020 which were non-cash in nature and represented the change in fair value of the preferred stock tranche liability associated with the Series A convertible preferred stock.

Net loss: Net loss for the year ended December 31, 2020 was $60.0 million, or $3.40 per share, as compared to a net loss of $1.1 million, or $0.12 per share, for the period from September 20, 2019 (the date of company inception) to December 31, 2019.

Cash and cash equivalents: As of December 31, 2020, Taysha had $251.3 million in cash and cash equivalents, which included $165.9 million in net proceeds from the company's IPO completed in September 2020.

Anticipated Milestones by Program

TSHA-101 for GM2 gangliosidosis: the first bicistronic gene therapy in clinical development designed to deliver two genes – *HEXA* and *HEXB* – intrathecally for the treatment of GM2 gangliosidosis, also called Tay-Sachs or Sandhoff disease

- Report preliminary Phase 1/2 safety and biomarker data (Queen's University trial) in the second half of 2021
- Submit an Investigational New Drug (IND) application in the U.S. in the second half of 2021
- Initiate Phase 1/2 clinical trial in the U.S. in the second half of 2021
- Report preliminary Phase 1/2 clinical data (Queen's University trial) by year-end 2021

TSHA-118 in CLN1: a self-complementary AAV9 viral vector designed to express a human codon-optimized CLN1 transgene to potentially treat CLN1, a rapidly progressing rare lysosomal storage disease with no approved treatments

- Maintain current open IND
- Initiate a Phase 1/2 clinical trial in the second half of 2021 with commercial-grade GMP material

TSHA-102 in Rett syndrome: a self-complementary AAV9 gene therapy in development for a severe neurodevelopmental disorder, designed to deliver MECP2 as well as a novel miRARE platform that regulates transgene expression on a cell-by-cell basis

- Submit IND/CTA filing in the second half of 2021
- Initiate Phase 1/2 clinical trial by year-end 2021 with commercial-grade GMP material

TSHA-104 in SURF1-associated Leigh syndrome: a self-complementary AAV9 viral vector with a codon optimized transgene encoding the human SURF1 protein to potentially treat SURF1-associated Leigh syndrome, a monogenic mitochondrial disorder with no approved treatments

- Submit IND/CTA filing in the second half of 2021
- Initiate Phase 1/2 trial by year-end 2021 with commercial-grade GMP material

Pipeline programs advancing into IND-/CTA-enabling studies

- Advance four programs into IND-/CTA-enabling studies including TSHA-105 in SLC13A5 deficiency, TSHA-111-LAFORIN and TSHA-111-MALIN in Lafora disease, TSHA-112 in APBD and TSHA-119 in GM2 AB variant, and continue IND-/CTA-enabling work on TSHA-103 in SLC6A1 haploinsufficiency disorder in 2021
- Submit an IND/CTA filing for one of the five above-named programs in 2021

Discovery programs

- Advance four new undisclosed programs focused on neurodevelopmental disorders, genetic epilepsies and neurodegenerative diseases into preclinical development in 2021

Next-generation technology platform

- Continue development efforts on regulated transgene expression with expansion of miRARE platform into additional CNS

diseases
- Explore vagus nerve redosing platform in large animal models
- Advance mini-gene discovery program in genetic forms of epilepsy and neurodevelopmental disorders
- Continue discovery and development efforts on next-generation capsids

Anticipated Corporate Milestones in 2021

- Initiate construction on internal cGMP facility in 2021
- Complete buildout of Dallas headquarters by mid-year
- Expand employee base from nearly 40 (as of December 31, 2020) to approximately 150 by year-end 2021

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 8:00 am ET / 7:00 am CT to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 833-614-1477 (U.S./Canada) or 914-987-7215 (international). The conference ID for all callers is 3183829. The live webcast and replay may be accessed by visiting Taysha's website at https://ir.tayshagtx.com/news-events/events-presentations. An archived version of the webcast will be available on the website for 30 days.

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 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 and early-stage programs, 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, our corporate growth plans and our plans to establish a commercial-scale cGMP manufacturing facility to provide preclinical, clinical and commercial supply. 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 Annual Report on Form 10-K for the year ended December 31, 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.

Taysha Gene Therapies, Inc.
Consolidated Statement of Operations
(in thousands, except share and per share data)

	For the Year Ended December 31, 2020	For the Period from September 20, 2019 (date of inception) to December 31, 2019
Operating expenses:		
Research and development	$ 31,893	$ 987
General and administrative	11,109	128
Total operating expenses	43,002	1,115
Loss from operations	(43,002)	(1,115)
Other (expense) income:		
Change in fair value of preferred stock tranche liability	(17,030)	—
Interest income	49	—
Interest expense	(28)	—
Total other (expense) income	(17,009)	—
Net loss	**$ (60,011)**	**$ (1,115)**
Net loss per common share, basic and diluted	$ (3.40)	$ (0.12)
Weighted average common shares outstanding, basic and diluted	17,665,683	9,625,679

Taysha Gene Therapies, Inc.
Consolidated Balance Sheet Data
(in thousands)

	December 31,	December 31,

	2020	2019
Cash and cash equivalents	$ 251,253	$ —
Total assets	$ 258,881	$ 15
Total liabilities	$ 7,579	$ 150
Total stockholders' equity (deficit)	$ 251,302	$ (135)

View source version on businesswire.com: https://www.businesswire.com/news/home/20210303005351/en/

Company Contact:
Kimberly Lee, D.O.
SVP, Corporate Communications and Investor Relations
Taysha Gene Therapies
klee@tayshagtx.com

Media Contact:
Carolyn Hawley
Canale Communications
carolyn.hawley@canalecomm.com

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