Taysha Gene Therapies Reports Second Quarter 2022 Financial Results and Provides Corporate Update

TSHA-120 treated patients in GAN demonstrated durable improvement and recoverability of sensory nerve amplitude potential (SNAP), a definitive clinical endpoint, compared to natural history

TSHA-120 commercial grade and clinical trial material considered comparable across all key quality attributes as assessed by an extensive panel of release assays and next-generation sequencing

Positive feedback from MHRA supports regulatory strategy and manufacturing approach including potency assay matrix; additional regulatory feedback, including from FDA, expected by year-end 2022

Preclinical data for TSHA-102 in Rett syndrome demonstrated near normalization of survival as well as normalization of behavior in neonatal knockout Rett mice; clinical data expected by year-end 2022

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

DALLAS, Aug. 11, 2022 (GLOBE NEWSWIRE) -- 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 reported financial results for the second quarter ended June 30, 2022, and provided a corporate update.

“We are excited to announce important progress in giant axonal neuropathy (GAN) including stabilization and improvement of sensory nerve function, a definitive clinical endpoint, in patients with GAN following treatment with TSHA-120,” said RA Session II, President, Founder and CEO of Taysha. “In addition, we now have positive comparability data demonstrating that our commercial grade and clinical trial material are comparable across all key quality attributes. Importantly, we believe positive feedback received from the MHRA, in conjunction with robust comparability data for TSHA-120 and comprehensive clinical data generated to date, further support our ongoing regulatory engagement. We expect additional regulatory feedback, including from the FDA, by year-end. In Rett syndrome, we are highly encouraged by late-breaking neonatal data in preclinical mouse models demonstrating near normalization of survival and normalization of behavior. We look forward to reporting preliminary Phase 1/2 clinical data in adult females with Rett syndrome by year-end 2022.”

Recent Corporate Highlights

TSHA-120 for giant axonal neuropathy (GAN): an intrathecally dosed AAV9 gene therapy in clinical development for the rare inherited genetic disorder GAN. TSHA-120 has received orphan drug and rare pediatric disease designations from the FDA and orphan drug designation from the European Commission.

- TSHA-120-treated patients with GAN demonstrated durable neurophysiological improvements in the sensory nerve action potential (SNAP), a definitive clinical endpoint, compared to rapid and irreversible decline in sensory function early in life in untreated patients based on natural history
    - Natural history data suggest that sensory function is unrecoverable once SNAP reaches zero
    - All patients who were 9 years and older in the natural history study demonstrated zero SNAP response
    - Treatment with TSHA-120 resulted in stabilization and improvement of sensory nerve function with durability of improvement in SNAP
- Pathology from nerve biopsies in all evaluable samples confirmed the presence of regenerative nerve fibers in 100% of TSHA-120-treated patients (n=11)
- TSHA-120 commercial grade and clinical trial material considered comparable across all key quality attributes as assessed by an extensive panel of release assays and next-generation sequencing
- Positive regulatory feedback from MHRA supports regulatory strategy
    - MHRA found functional clinical data, eye pathology and visual acuity, and nerve biopsy data compelling. Agency

agreed with commercial manufacturing and release testing strategy, including potency assays and recommended dosing a few patients with commercial grade material, which will be released in September 2022. MHRA was supportive of Taysha's proposal to perform validation work on MFM32 as a key clinical endpoint

TSHA-102 in Rett syndrome: a self-complementary intrathecally delivered AAV9 gene replacement therapy in clinical development for Rett syndrome. TSHA-102 utilizes the novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform to regulate transgene expression genotypically on a cell-by-cell basis. TSHA-102 is the first-and-only gene therapy in clinical development for Rett syndrome. TSHA-102 has received orphan drug and rare pediatric disease designations from the FDA and has been granted orphan drug designation from the European Commission.

- Late-breaking positive preclinical data for TSHA-102 in Rett syndrome demonstrated near normalization of survival and normalization of behavior in neonatal knockout Rett mice, a model significantly more severe than the human phenotype

Anticipated 2022 Milestones

- Further regulatory update, including from FDA, for TSHA-120 in GAN by year-end 2022
- First-in-human preliminary Phase 1/2 data for TSHA-102 in Rett syndrome by year-end 2022

Second Quarter 2022 Financial Highlights

Research and Development (R&D) Expenses: Research and development expenses were $23.1 million for the three months ended June 30, 2022, compared to $30.6 million for the three months ended June 30, 2021. The $7.5 million decrease was primarily attributable to a decrease of $3.8 million in third-party R&D, primarily related to GLP toxicology studies, a decrease of $3.2 million in R&D manufacturing costs, and lower employee compensation expenses of $0.5 million.

General and Administrative (G&A) Expenses: General and administrative expenses were $9.9 million for the three months ended June 30, 2022, compared to $10.1 million for the three months ended June 30, 2021. The decrease of approximately $0.2 million was primarily attributable to a decrease of $1.1 million in professional fees related to market research, recruiting, accounting, and patient advocacy activities. This was partially offset by $0.9 million of incremental employee compensation expenses.

Net loss: Net loss for the three months ended June 30, 2022, was $33.9 million, or $0.84 per share, as compared to a net loss of $40.9 million, or $1.09 per share, for the three months ended June 30, 2021.

Cash and cash equivalents: As of June 30, 2022, the Company had cash and cash equivalents of $66.2 million. Taysha continues to expect that its current cash and cash equivalents, in addition to full access to its existing term loan facility, is sufficient to fund operating expenses into the fourth quarter of 2023.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast with slides 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 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13730848. 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 goal 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," "plans," 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 our preclinical 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, 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, the potential market opportunity for these product candidates, our corporate growth plans, the forecast of our cash runway and the implementation and potential impacts of our strategic pipeline prioritization initiatives. 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 full-year ended December 31, 2021, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, both 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 disclaim any obligation to update these statements except as may be required by law.

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

(Unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 23,118	$ 30,643	$ 60,917	$ 54,497
General and administrative	9,867	10,129	21,336	18,365
Total operating expenses	32,985	40,772	82,253	72,862
Loss from operations	(32,985)	(40,772)	(82,253)	(72,862)
Other income (expense):				
Interest income	27	40	41	106
Interest expense	(912)	(194)	(1,761)	(194)
Other expense	(3)	-	(11)	-
Total other expense, net	(888)	(154)	(1,731)	(88)
Net loss	**$ (33,873)**	**$ (40,926)**	**$ (83,984)**	**$ (72,950)**
Net loss per common share, basic and diluted	$ (0.84)	$ (1.09)	$ (2.14)	$ (1.96)
Weighted average common shares outstanding, basic and diluted	40,142,403	37,479,164	39,163,996	37,237,115

Taysha Gene Therapies, Inc.
Condensed Consolidated Balance Sheet Data
(in thousands, except share and per share data)
(Unaudited)

	June 30, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 66,239	$ 149,103
Prepaid expenses and other current assets	10,596	10,499
Total current assets	76,835	159,602
Restricted cash	2,637	2,637
Deferred lease asset	643	667
Property, plant and equipment, net	61,011	50,610
Other non-current assets	1,206	440
Total assets	**$ 142,332**	**$ 213,956**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 23,967	$ 21,763
Accrued expenses and other current liabilities	18,986	29,983
Total current liabilities	42,953	51,746
Build-to-suit lease liability	25,609	25,900
Term Loan, net	37,580	37,192
Other non-current liabilities	3,480	3,735
Total liabilities	109,622	118,573
Stockholders' equity		
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of June 30, 2022 and December 31, 2021	-	-
Common stock, $0.00001 par value per share; 200,000,000 shares authorized and 41,020,086 and 38,473,945 issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	1	-
Additional paid-in capital	352,342	331,032
Accumulated deficit	(319,633)	(235,649)
Total stockholders' equity	32,710	95,383
Total liabilities and stockholders' equity	**$ 142,332**	**$ 213,956**

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