Taysha Gene Therapies Reports Third Quarter 2020 Financial Results and Provides Business Update

November 12, 2020

Successfully raised $96 million in Series B financing and completed $181 million IPO

Established management team with successful track record of developing and commercializing AAV9-based gene therapies for monogenic diseases of the central nervous system

Company on track to initiate a Phase 1/2 clinical trial of TSHA-101 in GM2 gangliosidosis by the end of 2020 and plans to submit four INDs in 2021

DALLAS--(BUSINESS WIRE)--Nov. 12, 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 reported its financial results for the third quarter of 2020 and provided a business update.

"Just over a year ago, we founded Taysha in partnership with UT Southwestern with a mission to develop gene therapies to address monogenic diseases of the central nervous system, and to that end we have made significant progress in building a talented team that can advance our broad portfolio," said RA Session, II, President, Founder and CEO of Taysha. "During the third quarter, we raised over $275 million between our Series B financing and our IPO, providing the capital resources to support our industry-leading pipeline of 18 gene therapy programs. We have also bolstered our GMP manufacturing capabilities to ensure we have the capacity to support our development plans for multiple programs by utilizing the UTSW vector core, our internal Taysha manufacturing facility that is currently in development and our newly announced partnership with Catalent."

Program Highlights

TSHA-101 for GM2 gangliosidosis – Taysha secured rare pediatric disease designation and orphan drug designation from the U.S. Food and Drug Administration (FDA) for TSHA-101, its bicistronic vector designed to treat GM2 gangliosidosis, also known as Tay-Sachs and Sandhoff disease. Taysha anticipates initiating a Phase 1/2 clinical trial of TSHA-101 in Canada by end of 2020.

TSHA-102 for Rett syndrome –Taysha announced that FDA has granted rare pediatric disease and orphan drug designation for TSHA-102, which is under development for the treatment of Rett syndrome, one of the most common genetic causes of severe intellectual disability. TSHA-102 utilizes the miRARE platform, which is designed to regulate the expression of therapeutic transgenes on a cellular basis. Taysha remains on track to file an Investigational New Drug (IND) application for the program in 2021.

TSHA-118 for CLN1 – Taysha announced the in-licensing of an AAV9-based gene therapy program for the treatment of CLN1. TSHA-118 was originally developed in the academic lab of Steven Gray, Ph.D., Taysha's Chief Scientific Advisor. CLN1 Batten disease is a rapidly progressive rare lysosomal storage disease with no approved treatments. The company intends to initiate a Phase 1/2 clinical trial of TSHA-118 in 2021 under a currently open IND.

Recent Business Highlights

In October, Taysha announced a partnership with Invitae to support two different screening programs to enable rapid identification of patients with genetic disorders. DETECT Lysosomal Storage Diseases provides genetic testing for lysosomal storage disorders, including GM2 gangliosidosis and CLN1. The Behind the Seizures® program supports the diagnosis of patients with genetic epilepsies.

In November, Taysha announced a development and manufacturing partnership with Catalent to support future preclinical and clinical supply for several of Taysha's gene therapy programs, including CLN1 and Rett syndrome.

Taysha has also invested in building its internal management team to advance the development of its broad gene therapy pipeline. The company anticipates more than tripling its headcount from its IPO by the end of 2020 across all areas of the organization.

Financial Results

As of September 30, 2020, cash and cash equivalents totaled $279 million, which includes gross proceeds of $181 million from Taysha's IPO and $96 million from its Series B financing. Taysha was incorporated in September of 2019 and thus has no previous nine-month results for comparison.

Taysha reported R&D expenses for the nine months ended September 30, 2020 of $19.6 million. The $19.6 million was primarily attributable to $10.0 million of expenses recognized pursuant to the Queen's University Agreement and the Abeona CLN1 Agreement, $4.1 million related to the manufacture of clinical trial material and $3.9 million in other sponsored research agreements for the Company's various product candidates. Additionally, Taysha incurred regulatory and clinical consulting expenses of $0.8 million and employee compensation and benefits expenses of $0.8 million, which included $0.4 million of non-cash stock-based compensation.

Taysha reported G&A expenses of $5.0 million for the nine months ended September 30, 2020. The $5.0 million was primarily attributable to $2.7 million of compensation and benefits related to new hires, which included $0.9 million of non-cash stock-based compensation, $1.7 million in consulting fees, $0.5 million of legal expenses related to general corporate matters and $0.1 million related to insurance and other administrative

expenses.

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," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning or implying the conduct or timing of our clinical trials and our research, development and regulatory plans for our product candidates, 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, 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 success of our partnerships with Invitae and Catalent. 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 we intend to file shortly hereafter and will be 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
Selected Condensed Consolidated Financial Information
(In thousands, except share and per share data)
(Unaudited)

Statements of Operations

	For the Three Months Ended September 30, 2020	For the Nine Months Ended September 30, 2020	For the Period from September 20, 2019 (date of inception) to September 30, 2019
Operating expenses:			
Research and development	$ 11,057	$ 19,633	$ -
General and administrative	3,984	5,002	31
Total operating expenses	15,041	24,635	31
Loss from operations	(15,041)	(24,635)	(31)
Other expense:			
Change in fair value of preferred stock tranche liability	-	(17,030)	-
Interest expense	(1)	(28)	-
Total other expense	(1)	(17,058)	-
Net loss	**$ (15,042)**	**$ (41,693)**	**$ (31)**
Net loss per common share, basic and diluted	$ (1.28)	$ (3.73)	$ (0.00)
Weighted average common shares outstanding, basic and diluted	11,733,170	11,176,429	8,715,999

Balance Sheet

	September 30, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 278,634	$ -
Prepaid expenses	604	-

Deferred offering costs	-	15
Total current assets	279,238	15
Property and equipment, net	28	-
Total assets	**$ 279,266**	**$ 15**
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities		
Accounts payable	$ 8,837	$ -
Accrued expenses	2,727	150
Due to related party	60	-
Total current liabilities	11,624	150
Total liabilities	11,624	150
Stockholders' equity (deficit)		
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of September 30, 2020; no shares authorized, issued and outstanding as of December 31, 2019	-	-
Common stock, $0.00001 par value per share; 200,000,000 shares authorized and 37,761,435 issued and outstanding as of September 30, 2020; 10,895,000 shares authorized, 10,894,999 issued and outstanding as of December 31, 2019	-	-
Additional paid-in capital	310,450	980
Accumulated deficit	(42,808)	(1,115)
Total stockholders'equity (deficit)	267,642	(135)
Total liabilities and stockholders' equity (deficit)	**$ 279,266**	**$ 15**

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