UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-39536

Taysha Gene Therapies, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**84-3199512**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
3000 Pegasus Park Drive Ste 1430 Dallas, Texas	**75247**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (214) 612-0000

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.00001 per share	TSHA	The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of May 14, 2024, the registrant had 187,018,275 shares of common stock, $0.00001 par value per share, outstanding.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	March 31, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 123,980	$ 143,940
Restricted cash	449	449
Prepaid expenses and other current assets	4,168	3,479
Assets held for sale	2,000	2,000
Total current assets	130,597	149,868
Restricted cash	2,151	2,151
Property, plant and equipment, net	10,686	10,826
Operating lease right-of-use assets	9,261	9,582
Other non-current assets	304	304
Total assets	**$ 152,999**	**$ 172,731**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 10,380	$ 6,366
Accrued expenses and other current liabilities	13,562	12,284
Deferred revenue	14,695	18,106
Total current liabilities	38,637	36,756
Term loan, net	40,512	40,508
Operating lease liability, net of current portion	18,499	18,953
Other non-current liabilities	1,502	1,577
Total liabilities	99,150	97,794
Commitments and contingencies - Note 13		
Stockholders' equity		
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of March 31, 2024 and December 31, 2023	—	—
Common stock, $0.00001 par value per share; 400,000,000 shares authorized and 187,018,275 and 186,960,193 issued and outstanding as of March 31, 2024 and December 31, 2023, respectively	2	2
Additional paid-in capital	591,166	587,942
Accumulated other comprehensive loss	(251)	—
Accumulated deficit	(537,068)	(513,007)
Total stockholders’ equity	53,849	74,937
Total liabilities and stockholders' equity	**$ 152,999**	**$ 172,731**

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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

	For the Three Months Ended March 31,	
	2024	**2023**
Revenue	$ 3,411	$ 4,706
Operating expenses:		
Research and development	20,657	12,514
General and administrative	7,084	8,751
Total operating expenses	27,741	21,265
Loss from operations	(24,330)	(16,559)
Other income (expense):		
Change in fair value of warrant liability	(337)	—
Change in fair value of term loan	(1,053)	—
Interest income	1,693	319
Interest expense	(29)	(1,374)
Other expense	(5)	(8)
Total other income (expense), net	269	(1,063)
Net loss	**$ (24,061)**	**$ (17,622)**
Net loss per common share, basic and diluted	$ (0.10)	$ (0.28)
Weighted average common shares outstanding, basic and diluted	231,249,344	63,260,905

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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

	For the Three Months Ended March 31,	
	2024	2023
Net loss	$ (24,061)	$ (17,622)
Other comprehensive loss:		
Change in fair value of terms loan attributable to instrument specific credit risk	(251)	—
Comprehensive loss	**$ (24,312)**	**$ (17,622)**

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statements of Stockholders' (Deficit) Equity
(in thousands, except share data)
(Unaudited)

For the Three Months Ended March 31, 2024

	Common Stock		Additional Paid-in	Accumulated	Accumulated Other	Total Stockholders' Equity
	Shares	Amount	Capital	Deficit	Comprehensive Loss	
Balance as of December 31, 2023	**186,960,193**	**$ 2**	**$ 587,942**	**$ (513,007)**	**$ —**	**$ 74,937**
Stock-based compensation	—	—	3,198	—	—	3,198
Issuance of common stock upon vesting and settlement of restricted stock units	11,282	—	—	—	—	—
Issuance of common stock under ESPP	46,800	—	26	—	—	26
Loss on instrument-specific credit risk	—	—	—	—	(251)	(251)
Net loss	—	—	—	(24,061)	—	(24,061)
Balance as of March 31, 2024	**187,018,275**	**$ 2**	**$ 591,166**	**$ (537,068)**	**$ (251)**	**$ 53,849**

For the Three Months Ended March 31, 2023

	Common Stock		Additional Paid-in	Accumulated	Accumulated Other	Total Stockholders' Equity
	Shares	Amount	Capital	Deficit	Comprehensive Loss	(Deficit)
Balance as of December 31, 2022	**63,207,507**	**$ 1**	**$ 402,389**	**$ (401,441)**	**$ —**	**$ 949**
Stock-based compensation	—	—	1,675	—	—	1,675
Issuance of common stock upon vesting and settlement of restricted stock units, net	229,922	—	—	—	—	—
Issuance of common stock under ESPP	35,920	—	50	—	—	50
Net loss	—	—	—	(17,622)	—	(17,622)
Balance as of March 31, 2023	**63,473,349**	**$ 1**	**$ 404,114**	**$ (419,063)**	**$ —**	**$ (14,948)**

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	For the Three Months Ended March 31,	
	2024	**2023**
Cash flows from operating activities		
Net loss	$ (24,061)	$ (17,622)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	325	331
Stock-based compensation	3,198	1,675
Change in fair value of warrant liability	337	—
Non-cash change in fair value of term loan	(247)	—
Non-cash lease expense	325	300
Other	27	128
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(693)	(400)
Accounts payable	3,980	359
Accrued expenses and other liabilities	422	(250)
Deferred revenue	(3,411)	(4,706)
Net cash used in operating activities	(19,798)	(20,185)
Cash flows from investing activities		
Purchase of property, plant and equipment	(140)	(3,900)
Net cash used in investing activities	(140)	(3,900)
Cash flows from financing activities		
Debt issuance costs for term loan	(18)	—
Payment of offering costs	—	(387)
Proceeds from common stock issuances under ESPP	26	50
Other	(30)	(33)
Net cash used in financing activities	(22)	(370)
Net decrease in cash, cash equivalents and restricted cash	(19,960)	(24,455)
Cash, cash equivalents and restricted cash at the beginning of the period	**146,540**	**90,517**
Cash, cash equivalents and restricted cash at the end of the period	**$ 126,580**	**$ 66,062**
Cash and cash equivalents	123,980	63,425
Restricted cash	2,600	2,637
Cash, cash equivalents and restricted cash at the end of the period	**$ 126,580**	**$ 66,062**
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 1,329	$ 1,125
Supplemental disclosure of noncash investing and financing activities:		
Property, plant and equipment in accounts payable and accrued expenses	52	112

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Note 1—Organization and Description of Business Operations

Taysha Gene Therapies, Inc. (the "Company" or "Taysha") was originally formed under the laws of the State of Texas on September 20, 2019. Taysha converted to a Delaware corporation on February 13, 2020, which had no impact to the Company's par value or issued and authorized capital structure.

Taysha is a clinical-stage biotechnology company focused on advancing AAV-based gene therapies for severe monogenic diseases of the central nervous system.

Sales Agreement

On October 5, 2021, the Company entered into a Sales Agreement (the "Sales Agreement") with SVB Securities LLC (f/k/a SVB Leerink LLC) and Wells Fargo Securities, LLC (collectively, the "Sales Agents"), pursuant to which the Company may issue and sell, from time to time in its sole discretion, shares of its common stock having an aggregate offering price of up to $150.0 million through the Sales Agents. In March 2022, the Company amended the Sales Agreement to, among other things, include Goldman Sachs & Co. LLC as an additional Sales Agent. The Sales Agents may sell common stock by any method permitted by law deemed to be an "at-the-market offering" as defined in Rule 415(a)(4) of the Securities Act, including sales made directly on or through the Nasdaq Global Select Market or any other existing trade market for the common stock, in negotiated transactions at market prices prevailing at the time of sale or at prices related to prevailing market prices, or any other method permitted by law. The Sales Agents are entitled to receive 3.0% of the gross sales price per share of common stock sold under the Sales Agreement. In April 2022, the Company sold 2,000,000 shares of common stock under the Sales Agreement and received $11.6 million in net proceeds. No other shares of common stock have been issued and sold pursuant to the Sales Agreement as of March 31, 2024.

Liquidity and Capital Resources

The Company has incurred operating losses since inception and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. Losses are expected to continue as the Company continues to invest in its research and development activities. As of March 31, 2024, the Company had an accumulated deficit of $537.1 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future.

Future capital requirements will depend on many factors, including the timing and extent of spending on research and development and the market acceptance of the Company's products. The Company will need to obtain additional financing in order to complete clinical studies and launch and commercialize any product candidates for which it receives regulatory approval. There can be no assurance that such financing will be available or will be on terms acceptable to the Company. As of March 31, 2024, the Company had cash and cash equivalents of $124.0 million, which the Company believes will be sufficient to fund its planned operations for a period of at least twelve months from the date of issuance of these unaudited condensed consolidated financial statements. The Company has based this estimate on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. The Company expects to finance its future cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its research and development programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

Note 2—Summary of Significant Accounting Policies

Basis of Presentation

The unaudited condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States ("GAAP") as determined by the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") for interim financial information and the instructions to Form 10-Q and Article 10 of Regulation S-X and are consistent in all material respects with those included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, filed with the Securities and Exchange Commission ("SEC") on March 19, 2024 (the "2023 Annual Report"). In the opinion of management, the unaudited condensed consolidated financial statements reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the balances and results for the periods presented. The condensed consolidated balance sheet as of December 31, 2023 is derived from audited financial statements, however, it does not include all of the information and footnotes required by GAAP for complete financial statements. These unaudited condensed consolidated financial statements should be read in conjunction with the consolidated financial statements and related notes in the Company's 2023 Annual Report.

Principles of Consolidation

The accompanying interim condensed consolidated financial statements include the accounts of Taysha and its wholly owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. The most significant estimates and assumptions in the Company's financial statements relate to the determination of the fair value of the common stock prior to the initial public offering ("IPO") (as an input into stock-based compensation), estimating manufacturing accruals and accrued or prepaid research and development expenses, the measurement of impairment of long-lived assets, the valuation of the Trinity Term Loans that are carried at fair value and the allocation of consideration received in connection with the Astellas Transactions (as defined below). These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Significant Accounting Policies

There have been no changes in the Company's significant accounting policies as disclosed in Note 2 to the audited consolidated financial statements included in the 2023 Annual Report.

Recently Adopted Accounting Pronouncements

There have been no significant changes in recently adopted accounting pronouncements from those disclosed in the section titled "Financial Statements and Supplementary Data" included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, as filed with the SEC.

Recently Issued Accounting Pronouncements

In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting – Improvements to Reportable Segment Disclosures*, to improve segment disclosure requirements under ASC 280, Segment Reporting, through enhancing disclosures about significant segment expenses. The guidance requires entities to provide significant segment expenses that are regularly provided to the chief operating decision maker and other segment expenses included in each reported measure of segment profitability. The ASU also enhances interim segment reporting requirements by aligning interim disclosures with information that must be disclosed annually in accordance with ASC 280. The guidance is effective for annual periods beginning after December 15, 2023, and interim periods

beginning after December 15, 2024, applied retrospectively with early adoption permitted. The Company is still evaluating the impact this ASU will have on its consolidated financial statements and related disclosures.

Note 3—Fair Value Measurements

The Company's financial instruments that are measured at fair value on a recurring basis consist of money market funds, the Trinity Term Loans, a success fee derivative liability and certain of the Company's warrant liabilities.

The following tables present information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy used to determine such fair values (in thousands):

	March 31, 2024			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents – money market funds	$ 122,916	$ 122,916	$ —	$ —
Total assets	$ 122,916	$ 122,916	$ —	$ —
Liabilities:				
Trinity Term Loans	$ 40,512	$ —	$ —	$ 40,512
Success Fee Derivative liability	826	—	—	826
SSI Warrant liability	791	—	—	791
Total liabilities	$ 42,129	$ —	$ —	$ 42,129

	December 31, 2023			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents – money market funds	$ 142,425	$ 142,425	$ —	$ —
Total assets	$ 142,425	$ 142,425	$ —	$ —
Liabilities:				
Trinity Term Loans	$ 40,508	$ —	$ —	$ 40,508
Success Fee Derivative liability	800	—	—	800
SSI Warrant liability	454	—	—	454
Total liabilities	$ 41,762	$ —	$ —	$ 41,762

The Company classifies its money market funds, which are valued based on quoted market prices in an active market with no valuation adjustment, as Level 1 assets within the fair value hierarchy.

The Company's Trinity Term Loans and Success Fee liability are classified as Level 3 measurements under the fair value hierarchy as the fair values were determined based on significant inputs not observable in the market. The fair values were determined utilizing a probability-weighted income approach, including variables for the timing of a success event and other probability estimates. See Note 7 for additional information on the Trinity Term Loans and Success Fee.

The Company's SSI Warrant liability is classified as Level 3 measurements under the fair value hierarchy as the fair values were determined based on significant inputs not observable in the market. The fair values were determined using the Black-Scholes-Merton option pricing model to determine the fair value of the SSI Warrants (as defined below). See Note 10 for additional information on the SSI Warrants.

Note 4—Balance Sheet Components

Prepaid expenses and other current assets consisted of the following (in thousands):

	March 31, 2024	December 31, 2023
Prepaid research and development	$ 1,921	$ 1,412
Prepaid clinical trial	955	802
Deferred offering costs	681	681
Prepaid insurance	269	292
Prepaid bonus	193	—
Other	149	292
Total prepaid expenses and other current assets	$ 4,168	$ 3,479

Property, plant and equipment, net consisted of the following (in thousands):

	March 31, 2024	December 31, 2023
Leasehold improvements	$ 2,117	$ 2,117
Laboratory equipment	3,008	2,868
Computer equipment	1,133	1,133
Furniture and fixtures	864	864
Construction in progress	6,875	6,823
	13,997	13,805
Accumulated depreciation	(3,311)	(2,979)
Property, plant and equipment, net	$ 10,686	$ 10,826

Property, plant and equipment, net includes $0.9 million and $1.0 million of assets capitalized as finance leases as of March 31, 2024 and December 31, 2023, respectively.

Depreciation expense was $0.3 million for each of the three months ended March 31, 2024 and 2023, respectively.

Accrued expenses and other current liabilities consisted of the following (in thousands):

	March 31, 2024	December 31, 2023
Accrued research and development	$ 6,078	$ 3,467
Lease liabilities, current portion	1,696	1,646
Accrued clinical trial	1,598	1,851
Accrued compensation	1,576	3,423
Accrued professional and consulting fees	1,014	330
Warrant liability	791	454
Accrued severance	28	390
Other	781	723
Total accrued expenses and other current liabilities	$ 13,562	$ 12,284

Note 5— Leases

The Company leases certain office, laboratory, and manufacturing space.

Dallas Lease

On January 11, 2021, the Company entered into a lease agreement (the "Dallas Lease") with Pegasus Park, LLC, a Delaware limited liability company (the "Dallas Landlord"), pursuant to which the Company will lease approximately 15,000 square feet of office space at 3000 Pegasus Park Drive, Dallas, Texas 75247 (the "Office Space").

The Dallas Lease commenced on May 27, 2021, and has a term of approximately ten years. The Company has an option to extend the term of the Dallas Lease for one additional period of five years.

The Dallas Landlord has the right to terminate the Dallas Lease, or the Company's right to possess the Office Space without terminating the Dallas Lease, upon specified events of default, including the Company's failure to pay rent in a timely manner and upon the occurrence of certain events of insolvency with respect to the Company.

Dallas Lease Expansion

On December 14, 2021, the Company amended the Dallas Lease (the "Dallas Lease Amendment") with the Dallas Landlord, pursuant to which the Company will lease approximately 18,000 square feet of office space adjacent to the Office Space at 3000 Pegasus Park Drive, Dallas, Texas 75247 (the "Expansion Premises").

The Dallas Lease Amendment commenced on July 1, 2022, and has a term of approximately ten years.

The Company is obligated to pay operating costs and utilities applicable to the Expansion Premises. Total future minimum lease payments under the Dallas Lease Amendment over the initial 10 year term are approximately $6.0 million. The Company will be responsible for costs of constructing interior improvements within the Expansion Premises that exceed a $40.00 per rentable square foot construction allowance provided by the Dallas Landlord.

The Company has a right of first refusal with respect to certain additional office space on the 15th floor at 3000 Pegasus Park Drive, Dallas, Texas 75247 before the Dallas Landlord accepts any offer for such space.

Durham Lease

On December 17, 2020, the Company entered into a lease agreement (the "Durham Lease") with Patriot Park Partners II, LLC, a Delaware limited liability company (the "Durham Landlord"), pursuant to which the Company agreed to lease approximately 187,500 square feet of a manufacturing facility located at 5 National Way, Durham, North Carolina (the "Facility"). The Durham Lease commenced on April 1, 2021 and is expected to have a term of approximately fifteen years and six months. The Company has two options to extend the term of the Durham Lease, each for a period of an additional five years.

The Company was not required to provide a security deposit in connection with its entry into the Durham Lease. The Company was responsible for constructing interior improvements within the Facility. The Company was required to place $2.6 million in an escrow account which was to be released when the improvements were substantially complete. In December 2023, the Company entered into an agreement with the landlord whereby the Company agreed to remove specified leasehold improvements which will be funded by the escrowed funds. The escrow funds are recorded as restricted cash on the condensed consolidated balance sheets as of March 31, 2024 with $0.5 million recorded in current assets and $2.1 million in noncurrent assets. The Durham Landlord has the right to terminate the Durham Lease upon specified events of default, including the Company's failure to pay rent in a timely manner and upon the occurrence of certain events of insolvency with respect to the Company.

Summary of all lease costs recognized under ASC 842

The following table summarizes the lease costs recognized under ASC 842 and other information pertaining to the Company's operating leases for the three months ended March 31, 2024 and 2023 (in thousands):

	For the Three Months Ended March 31,	
	2024	**2023**
Operating lease cost	$ 646	$ 708
Variable lease cost	198	243
Total lease cost	$ 844	$ 951

Supplemental information related to the remaining lease term and discount rate are as follows:

	March 31, 2024	December 31, 2023
Weighted average remaining lease term (in years) – Finance leases	2.63	2.88
Weighted average remaining lease term (in years) – Operating leases	10.55	10.75
Weighted average discount rate – Finance leases	10.52%	10.52%
Weighted average discount rate – Operating leases	7.81%	7.80%

Supplemental cash flow information related to the Company's operating leases are as follows (in thousands):

	For the Three Months Ended March 31,	
	2024	2023
Operating cash flows for operating leases	$ 2,742	$ 692

As of March 31, 2024, future minimum commitments under ASC 842 under the Company's operating and finance leases were as follows (in thousands):

Year Ending December 31,	Operating	Finance
2024	$ 2,123	$ 341
2025	2,910	454
2026	2,485	399
2027	2,577	—
2028	2,673	—
Thereafter	17,045	—
Total lease payments	29,813	1,194
Less: imputed interest	(9,980)	(156)
Total lease liabilities	$ 19,833	$ 1,038
Lease liabilities, current	1,334	362
Lease liabilities, non-current	18,499	676
Total lease liabilities	$ 19,833	$ 1,038

Note 6—Astellas Agreements

On October 21, 2022 (the "Effective Date"), the Company entered into the Option Agreement (the "Option Agreement") with Audentes Therapeutics, Inc. (d/b/a Astellas Gene Therapy)("Astellas"), pursuant to which the Company granted to Astellas an exclusive option to obtain an exclusive, worldwide, royalty and milestone-bearing right and license (A) to research, develop, make, have made, use, sell, offer for sale, have sold, import, export and otherwise exploit, or, collectively, exploit, the product known, as of the Effective Date, as TSHA-120 (the "120 GAN Product"), and any backup products with respect thereto for use in the treatment of Giant Axonal Neuropathy ("GAN") or any other gene therapy product for use in the treatment of GAN that is controlled by Taysha or any of its affiliates or with respect to which the Company or any of its affiliates controls intellectual property rights covering the exploitation thereof, or a GAN Product, and (B) under any intellectual property rights controlled by Taysha or any of its affiliates with respect to such exploitation (the "GAN Option"). Subject to certain extensions, the GAN Option was exercisable from the Effective Date through a specified period of time following Astellas' receipt of (i) the formal minutes from the Type B end-of-Phase 2 meeting between Taysha and the FDA in response to the Company's meeting request sent to the FDA on September 19, 2022 for the 120 GAN Product (the "Type B end-of-Phase 2 Meeting"), (ii) all written feedback from the FDA with respect to the Type B end-of-Phase 2 Meeting, and (iii) all briefing documents sent by Taysha to the FDA with respect to the Type B end-of-Phase 2 Meeting. In September 2023, Astellas provided written notice of its decision not to exercise the GAN Option.

Under the Option Agreement, the Company also granted to Astellas an exclusive option to obtain an exclusive, worldwide, royalty and milestone-bearing right and license (A) to exploit any Rett Product (as defined below), and (B) under any intellectual property rights controlled by Taysha or any of its affiliates with respect to such exploitation (the "Rett Option," and together with the GAN Option, each, an "Option"). Subject to certain extensions, the Rett Option is exercisable from the Effective Date through a specified period of time following Astellas' receipt of (i) certain clinical data from the female pediatric trial and (ii) certain specified data with respect to TSHA-102, such period, the Rett Option Period, related to (i) the product known, as of the Effective Date, as

TSHA-102 and any backup products with respect thereto for use in the treatment of Rett syndrome, and (ii) any other gene therapy product for use in the treatment of Rett syndrome that is controlled by Taysha or any of its affiliates or with respect to which the Company or any of its affiliates controls intellectual property rights covering the exploitation thereof (a "Rett Product").

The parties have agreed that, if Astellas exercises an Option, the parties will, for a specified period, negotiate a license agreement in good faith on the terms and conditions outlined in the Option Agreement, including payments by Astellas of a to be determined upfront payment, certain to be determined milestone payments, and certain to be determined royalties on net sales of GAN Products and/or Rett Products, as applicable.

During the Rett Option Period, the Company has agreed to (A) not solicit or encourage any inquiries, offers or proposals for, or that could reasonably be expected to lead to, a Change of Control (as defined in the Option Agreement), or (B) otherwise initiate a process for a potential Change of Control, in each case, without first notifying Astellas and offering Astellas the opportunity to submit an offer or proposal to the Company for a transaction that would result in a Change of Control. If Astellas fails or declines to submit any such offer within a specified period after the receipt of such notice, the Company will have the ability to solicit third party bids for a Change of Control transaction. If Astellas delivers an offer to the Company for a transaction that would result in a Change of Control, the Company and Astellas will attempt to negotiate in good faith the potential terms and conditions for such potential transaction that would result in a Change of Control for a specified period, which period may be shortened or extended by mutual agreement.

As partial consideration for the rights granted to Astellas under the Option Agreement, Astellas paid the Company an upfront payment of $20.0 million (the "Upfront Payment"). Astellas or any of its affiliates shall have the right, in its or their discretion and upon written notice to the Company, to offset the amount of the Upfront Payment (in whole or in part, until the full amount of the Upfront Payment has been offset) against (a) any payment(s) owed to Taysha or any of its affiliates (or to any third party on behalf of the Company) under or in connection with any license agreement entered into with respect to any GAN Product or Rett Product, including, any upfront payment, milestone payment or royalties owed to Taysha or any of its affiliates (or to any third party on behalf of the Company) under or in connection with any such license agreement or (b) any amount owed to Taysha or any of its affiliates in connection with a Change of Control transaction with Astellas or any of its affiliates. As further consideration for the rights granted to Astellas under the Option Agreement, the Company and Astellas also entered into the Astellas Securities Purchase Agreement (as defined below).

Astellas Securities Purchase Agreement

On October 21, 2022, the Company entered into a securities purchase agreement with Astellas (the "Astellas Securities Purchase Agreement"), pursuant to which the Company agreed to issue and sell to Astellas in a private placement (the "Astellas Private Placement"), an aggregate of 7,266,342 shares (the "Astellas Private Placement Shares"), of its common stock, for aggregate gross proceeds of $30.0 million. The Astellas Private Placement closed on October 24, 2022. Pursuant to the Securities Purchase Agreement, in connection with the Astellas Private Placement, Astellas has the right to designate one individual to attend all meetings of the Board in a non-voting observer capacity. The Company also granted Astellas certain registration rights with respect to the Astellas Private Placement Shares.

Accounting Treatment

In October 2022, upon closing of the Astellas Private Placement and transferring the 7,266,342 shares to Astellas, the Company recorded the issuance of shares at fair value. Fair value of the shares transferred to Astellas was calculated in accordance with ASC 820, *Fair Value Measurement* by analyzing the Company's stock price for a short period of time prior to and after the transaction date as traded on the NASDAQ. The NASDAQ trading data is considered an active market and a Level 1 measurement under ASC 820. The fair value was determined to be approximately $13.95 million or $1.92 per share. The $16.1 million difference between the $30.0 million paid by Astellas and the fair market value of shares issued was allocated to the transaction price of the Option Agreement.

The Company determined that the Option Agreement falls within the scope of ASC 606, *Revenue from Contracts with Customers* as the development of TSHA-102 for the treatment of Rett Syndrome and TSHA-120 for the treatment of GAN are considered ordinary activities for the Company. In accordance with ASC 606, the Company evaluated the Option Agreement and identified three separate performance obligations: (1) option to obtain licensing right to GAN, (2) option to obtain licensing right to Rett and (3) performance of research and development activities in the Rett development plan. The transaction price is determined to be $36.1 million which is comprised of the $20.0 million Upfront Payment and the $16.1 million allocated from the Private Placement.

To determine the standalone selling price ("SSP") of the Rett and GAN options, which the Company concluded to be material rights, the Company utilized the probability-weighted expected return (PWERM) method. The PWERM method contemplates the

probability and timing of an option exercise. At contract inception, the Company estimated that the probability of exercise was 50% for each of the GAN and Rett options. The SSP of the Rett research and development activities was estimated using an expected cost plus margin approach. The standalone selling prices of the material rights and Rett research and development activities were then used to proportionately allocate the $36.1 million transaction price to the three performance obligations. The $36.1 million transaction price was recorded as deferred revenue on the condensed consolidated balance sheet at the inception of the Astellas Transactions.

The following table summarizes the allocation of the transaction price to the three performance obligations at contract inception (amounts in thousands):

	Transaction Price Allocation
Option to obtain license for Rett	$ 5,485
Option to obtain license for GAN	2,317
Rett research and development activities	28,257
Total	$ 36,059

Revenue allocated to the material rights will be recognized at a point in time when each option period expires or when a decision is made by Astellas to exercise or not exercise each option. Revenue from the Rett research and development activities will be recognized as activities are performed using an input method, according to the costs incurred as related to the total costs expected to be incurred to satisfy the performance obligation. The transfer of control occurs over this time period and is a reliable measure of progress towards satisfying the performance obligation.

During the three months ended March 31, 2024, there were no significant changes to the total estimated costs to be incurred to satisfy the performance obligation associated with the Rett research and development activities.

The Company recognized revenue of $3.4 million and $4.7 million from Rett research and development activities for the three months ended March 31, 2024 and 2023, respectively.

The Company had $14.7 million of deferred revenue on the condensed consolidated balance sheet as of March 31, 2024 comprised of $5.5 million for the Rett Option and $9.2 million of Rett research and development activities. The GAN option revenue was recognized in September 2023 when Astellas provided written notice of its decision not to exercise the GAN Option.

Note 7 – Term Loans

Loan with Trinity Capital

On November 13, 2023 (the "Trinity Closing Date"), the Company entered into a Loan and Security Agreement (the "Trinity Term Loan Agreement"), by and among the Company, the lenders party thereto from time to time (the "Trinity Lenders") and Trinity Capital Inc., as administrative agent and collateral agent for the Trinity Lenders ("Trinity"). The Trinity Term Loan Agreement provides for, on the Trinity Closing Date, $40.0 million aggregate principal amount of term loans (collectively, the "Trinity Term Loans"). The Company drew the Trinity Term Loans in full on the Trinity Closing Date.

The interest rate applicable to the Trinity Term Loans is the greater of (a) the Wall Street Journal ("WSJ") Prime Rate plus 4.50% or (b) 12.75% per annum. The Trinity Term Loans are interest only from the Trinity Closing Date through 36 months from the Trinity Closing Date, which may be extended to 48 months from the Trinity Closing Date upon the satisfaction of certain milestones set forth in the Trinity Term Loan Agreement, after which the Company is required to pay equal monthly installments of principal through November 13, 2028 (the "Maturity Date"). As of March 31, 2024, $40.0 million was outstanding on the Term Loan, recorded as Term Loan, net on the condensed consolidated balance sheet.

Future principal debt payments on the Term Loan Agreement as of March 31, 2024 are as follows (in thousands):

Year Ending December 31,	
2024	$ —
2025	—
2026	—
2027	18,709
2028	21,291
Total principal payments	$ 40,000

The Trinity Term Loans may be prepaid in full (i) from the Trinity Closing Date through November 13, 2024, with payment of a 3.00% prepayment premium, (ii) from November 13, 2024 through November 13, 2025, with payment of a 2% prepayment premium, and (iii) from November 13, 2025 through, but excluding, the Maturity Date, with payment of a 1% prepayment premium. On the Trinity Closing Date, the Company paid to Trinity a commitment fee of 1.00% of the original principal amount of the Trinity Term Loans. Upon repayment in full of the Trinity Term Loans, the Company will pay to Trinity an end of term payment equal to 5.00% of the original principal amount of the Trinity Term Loans.

The obligations under the Trinity Term Loan Agreement are secured by a perfected security interest in all of the Company's assets except for certain customarily excluded property pursuant to the terms of the Trinity Term Loan Agreement. There are no financial covenants and no warrants associated with the Trinity Term Loan Agreement. The Trinity Term Loan Agreement contains various covenants that limit the Company's ability to engage in specified types of transactions without the consent of Trinity and the Trinity Lenders which include, among others, incurring or assuming certain debt; merging, consolidating or acquiring all or substantially all of the capital stock or property of another entity; changing the nature of the Company's business; changing the Company's organizational structure or type; licensing, transferring or disposing of certain assets; granting certain types of liens on the Company's assets; making certain investments; and paying cash dividends. As of March 31, 2024, the Company is in compliance with all covenants of the Trinity Term Loans.

The Trinity Term Loan Agreement also contains customary representations and warranties, and also includes customary events of default, including payment default, breach of covenants, change of control, and material adverse effects. Upon the occurrence of an event of default, a default interest rate of an additional 5% per annum may be applied to the outstanding loan balances, and the Trinity Lenders may declare all outstanding obligations immediately due and payable and exercise all of its rights and remedies as set forth in the Trinity Term Loan Agreement and under applicable law.

The proceeds of the Trinity Term Loans were used to repay the Company's obligations under the Term Loan Agreement with Silicon Valley Bank in full. The Term Loan Agreement with Silicon Valley Bank was terminated concurrently with entry into the Trinity Term Loan Agreement.

The Company assessed the terms and features of the Trinity Term Loans and determined that the Company was eligible to elect the fair value option under ASC 825, *Financial Instruments*. The Trinity Term Loans contain various embedded features and the election of the fair value option allowed the Company to bypass analysis of potential embedded derivatives and further analysis of bifurcation of any recognized financial liabilities. Under the fair value option, the financial liability is initially measured at its fair value on the issue date and subsequently remeasured at estimated fair value on a recurring basis at each reporting date. Changes in the fair value of the Trinity Term Loans, which include accrued interest, if any, are recorded as a component of other expense (income) in the condensed consolidated statements of operations. The Company has not elected to present interest expense separately from changes in fair value and therefore will not present interest expense associated with the Trinity Term Loans. Any changes in fair value caused by instrument-specific credit risk are presented separately in other comprehensive income or loss if material. Under the fair value option, debt issuance costs are expensed as incurred. The Company incurred $0.9 million of debt issuance costs, which were recorded within general and administrative expense in the consolidated statements of operations for the year ended December 31, 2023.

In connection with the Trinity Term Loans, the Company entered into a Success Fee Agreement with Trinity which specifies the terms regarding a fee in the amount of 10% of the principal amount of the funded Trinity Term Loans (the "Success Fee"). The Success Fee is payable upon the achievement of certain corporate development value-inflection milestones. The Success Fee survives the termination of the Trinity Term Loans and expires on the earlier of ten years, or payment in full in cash of the Success Fee. The Company determined that the Success Fee represents a freestanding financial instrument and should be accounted for as a derivative liability under ASC 815 and recorded a liability within other non-current liabilities on the consolidated balance sheet, at fair value on the Trinity Closing Date and will be marked-to-market at the end of each reporting period with gains and losses recognized as a component of other income (expense) in the condensed consolidated statements of operations.

The proceeds from the Trinity Term Loans were allocated to the Success Fee and Trinity Term Loans based on their respective fair values on the Trinity Closing Date. The fair values were determined utilizing a probability-weighted income approach, including variables for the timing of a success event and other probability estimates.

The Company determined the fair value of the Trinity Term Loans and the Success Fee using a probability-weighted income approach and recorded the loan at fair value of $39.2 million and the Success Fee liability at fair value of $0.8 million in the condensed consolidated balance sheet at issuance. The Company calculated the discounted cash flows of the Trinity Term Loans using a discount rate of 15.68% and adjusted for the probability of various repayment scenarios. The Company calculated the discounted cash flows of the Success Fee liability, using a discount rate of 15.68% then adjusted for the probability of achievement of certain corporate development value-inflection milestones.

The Company remeasured the fair value of the Trinity Term Loans and Success Fee as of March 31, 2024 using a probability-weighted income approach. The Company calculated discounted cash flows of the Trinity Term Loans using a discount rate of 15.00% and adjusted for the probability of various repayment scenarios. The Company calculated the discounted cash flows of the Success Fee liability, using a discount rate of 15.00% then adjusted for the probability of achievement of certain corporate development value-inflection milestones.

The following table reconciles the change in fair value of the Trinity Term Loans during the three months ended March 31, 2024 (in thousands):

Trinity Term Loans		
Beginning fair value balance at January 1, 2024	$	40,508
Principal payments		—
Change in fair value reported in statements of operations		(247)
Change in fair value reported in comprehensive loss		251
Ending fair value balance as of March 31, 2024	$	40,512

During the three months ended March 31, 2024, the Company recorded $1.3 million of interest expense within change in fair value of term loans, all of which was paid as of March 31, 2024.

The following table reconciles the change in fair value of the Success Fee liability during the three months ended March 31, 2024 (in thousands):

Success Fee		
Beginning fair value balance at January 1, 2024	$	800
Change in fair value of Success Fee		26
Ending fair value balance as of March 31, 2024	$	826

Loan with Silicon Valley Bank

On August 12, 2021 (the "Closing Date"), the Company entered into a Loan and Security Agreement (the "Term Loan Agreement"), by and among the Company, the lenders party thereto from time to time (the "Lenders") and Silicon Valley Bank, as administrative agent and collateral agent for the Lenders ("Agent"). The Term Loan Agreement provided for (i) on the Closing Date, $40.0 million aggregate principal amount of term loans available through December 31, 2021, (ii) from January 1, 2022 until September 30, 2022, an additional $20.0 million term loan facility available at the Company's option upon having three distinct and active clinical stage programs, determined at the discretion of the Agent, at the time of draw, (iii) from October 1, 2022 until March 31, 2023, an additional $20.0 million term loan facility available at the Company's option upon having three distinct and active clinical stage programs, determined at the discretion of the Agent, at the time of draw and (iv) from April 1, 2023 until December 31, 2023, an additional $20.0 million term loan facility available upon approval by the Agent and the Lenders (collectively, the "Term Loans"). The Company drew $30.0 million in term loans on the Closing Date and $10.0 million in term loans in December 2021. The Company did not draw on the two additional $20.0 million tranches prior to expiration on September 30, 2022 and March 31, 2023. The Term Loan Agreement with Silicon Valley Bank was terminated concurrently with entry into the Trinity Term Loan Agreement in November 2023.

The interest rate applicable to the Term Loans was the greater of (a) the WSJ Prime Rate plus 3.75% or (b) 7.00% per annum. The Term Loans were interest only from the Closing Date through August 31, 2024, after which the Company was required to pay equal monthly installments of principal through August 1, 2026, the maturity date.

The Term Loans could have been prepaid in full through August 12, 2023, with payment of a 1.00% prepayment premium, after which they could be prepaid in full with no prepayment premium. An additional final payment of 7.5% of the amount of Terms Loans advanced by the Lenders ("Exit Fee") was due upon prepayment or repayment of the Term Loans in full. The Exit Fee of $3.0 million was recorded as debt discount. The debt discount was being accreted using the effective interest method over the term of the Term Loans within interest expense in the condensed consolidated statements of operations.

The obligations under the Term Loan Agreement were secured by a perfected security interest in all of the Company's assets except for intellectual property and certain other customarily excluded property pursuant to the terms of the Term Loan Agreement.

Upon termination of the Term Loan Agreement with Silicon Valley Bank, the Company made a prepayment of $43.2 million to satisfy the Company's principal and interest obligations and related fees under the Term Loan Agreement. The payoff amount paid

by the Company in connection with the Term Loans included payment of the Exit Fee of $3.0 million and accrued interest of $0.2 million. In connection with the repayment of the Term Loans, the remaining balance of debt discount of $1.4 million was recognized as a component of other income (expense) in the condensed consolidated statements of operations for the year ended December 31, 2023.

During the three months ended March 31, 2023, the Company recognized interest expense related to the Term Loans of $1.3 million.

Note 8—Research, Collaboration and License Agreements

UT Southwestern Agreement

On November 19, 2019, the Company entered into a research, collaboration and license agreement ("UT Southwestern Agreement") with the Board of Regents of the University of Texas System on behalf of The University of Texas Southwestern Medical Center ("UT Southwestern"). Under the UT Southwestern Agreement, UT Southwestern is primarily responsible for preclinical development activities with respect to licensed products for use in certain specified indications (up to investigational new drug application-enabling studies), and the Company is responsible for all subsequent clinical development and commercialization activities with respect to the licensed products. UT Southwestern will conduct such preclinical activities for a two-year period under mutually agreed upon sponsored research agreements that were entered into beginning in April 2020. During the initial research phase, the Company has the right to expand the scope of specified indications under the UT Southwestern Agreement.

In connection with the UT Southwestern Agreement, the Company obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, the Company obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights. The Company is required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

On April 2, 2020, the Company amended the UT Southwestern Agreement to include the addition of another licensed product and certain indications, and a right of first refusal to the Company over certain patient dosing patents. No additional consideration was transferred in connection with this amendment. In March 2022, the Company and UT Southwestern mutually agreed to revise the payment schedules and current performance expectations of the current sponsored research agreements under the UT Southwestern Agreement and defer payments by fifteen months. In December 2023, the Company and UT Southwestern mutually agreed to terminate specific sponsored research agreements.

The UT Southwestern Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last valid claim of a licensed patent in such country for such licensed product. After the initial research term, the Company may terminate the agreement, on an indication-by-indication and licensed product-by-licensed product basis, at any time upon specified written notice to UT Southwestern. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. In December 2023, the Company transferred rights to specific indications back to UT Southwestern.

In November 2019, as partial consideration for the license rights granted under the UT Southwestern Agreement, the Company issued 2,179,000 shares of its common stock, or 20% of its then outstanding fully-diluted common stock, to UT Southwestern. The Company does not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement other than costs related to maintenance of patents.

Queen's Agreement

On February 21, 2020, the Company entered into a license agreement with Queen's (the "Queen's Agreement") to obtain the exclusive perpetual, royalty-bearing license, with the right to sublicense through multiple tiers, under certain patent rights and know-how of Queen's, including certain improvements to such patent rights and know-how, to develop products in any field which use one or more valid claims of the patents licensed under the Queen's Agreement (the "Licensed Patents"), or the technology, information and intellectual property related to the patents licensed under the Queen's Agreement (together with the Licensed Patents, the "Licensed Products"), and to make, have made, use, sell, offer for sale, import and export Licensed Products and otherwise exploit such patents and know-how for use in certain specified indications. In exchange for the rights granted to the Company, the Company made a cash payment of $3.0 million in April 2020 which was recorded within research and development expenses in the consolidated statements of operations since the acquired license does not have an alternative future use. The Company is obligated to make aggregate cash payments of up to $10.0 million upon the completion of a combination of regulatory milestones and up to $10.0

million upon the completion of a combination of commercial milestones. In further consideration of the rights granted, beginning with the Company's first commercial sale of the Licensed Products, the Company will also pay an annual earned royalty in the low single digits on net sales of Licensed Products, subject to certain customary reductions, and a percentage of non-royalty sublicensing revenue ranging in the low double digits. Royalties are payable, on a Licensed Products-by-Licensed Products and a country-by-country basis, until expiration of the last valid claim of a Licensed Patent covering such Licensed Products in such country and the expiration of any regulatory exclusivity for such Licensed Products in such country.

In January 2024, the Company transferred rights back to Queen's for the Licensed Patents. No additional milestone payments were made in connection with the Queen's Agreement during the three months ended March 31, 2024.

Abeona CLN1 Agreements

In August 2020, the Company entered into license and inventory purchase agreements with Abeona Therapeutics Inc. ("Abeona") for worldwide exclusive rights to certain intellectual property rights and know-how relating to the research, development and manufacture of ABO-202, an AAV-based gene therapy for CLN1 disease (also known as infantile Batten disease). Under the terms of the agreements, the Company made initial cash payments to Abeona of $3.0 million for the license fee and $4.0 million for purchase of clinical materials and reimbursement for previously incurred development costs in October 2020. In exchange for the license rights, the Company recorded an aggregate of $7.0 million within research and development expenses in the consolidated statements of operations for the year ended December 31, 2020 since the acquired license or acquired inventory do not have an alternative future use. The Company is obligated to make up to $26.0 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed CLN1 product. The Company will also pay an annual earned royalty in the high single digits on net sales of any licensed CLN1 products. The license agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. The Company may terminate the license agreement for convenience upon specified prior written notice to Abeona.

In December 2021, a regulatory milestone was triggered in connection with this agreement and therefore the Company recorded $3.0 million within research and development expenses in the consolidated statements of operations for the year ended December 31, 2021. The milestone fee was paid in January 2022 and classified as an investing cash outflow in the condensed consolidated statements of cash flows. No additional milestone payments were triggered in connection with this agreement during the three months ended March 31, 2024.

Abeona Rett Agreement

On October 29, 2020, the Company entered into a license agreement (the "Abeona Rett Agreement") with Abeona pursuant to which the Company obtained an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses under certain patents, know-how and materials originally developed by the University of North Carolina at Chapel Hill, the University of Edinburgh and Abeona to research, develop, manufacture, have manufactured, use, and commercialize licensed products for gene therapy and the use of related transgenes for Rett syndrome.

Subject to certain obligations of Abeona, the Company is required to use commercially reasonable efforts to develop at least one licensed product and commercialize at least one licensed product in the United States.

In connection with the Abeona Rett Agreement, the Company paid Abeona a one-time upfront license fee of $3.0 million which was recorded in research and development expenses in the consolidated statements of operations for the year ended December 31, 2020 since the acquired license does not have an alternative future use. The Company is obligated to pay Abeona up to $26.5 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed Rett product and high single-digit royalties on net sales of licensed Rett products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the latest of the expiration or revocation or complete rejection of the last licensed patent covering such licensed product in the country where the licensed product is sold, the loss of market exclusivity in such country where the product is sold, or, if no licensed product exists in such country and no market exclusivity exists in such country, ten years from first commercial sale of such licensed product in such country.

The Abeona Rett Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. The Company may terminate the agreement for convenience upon specified prior written notice to Abeona.

In March 2022, the Company's clinical trial application, ("CTA") filing for TSHA-102 for the treatment of Rett Syndrome was approved by Health Canada and therefore triggered a regulatory milestone payment in connection with this agreement. The

Company recorded $1.0 million within research and development expenses and classified the payment as an investing cash outflow in the consolidated statements of cash flows. In May 2023, the Company dosed the first patient with TSHA-102 in the Phase 1/2 REVEAL trial evaluating the safety and preliminary efficacy of TSHA-102 in adult patients with Rett syndrome and therefore triggered a milestone payment in connection with the Abeona Rett Agreement. The Company recorded $3.5 million within research and development expenses in the condensed consolidated statements of operations for the year ended December 31, 2023. This milestone fee was paid in August 2023 and classified as an investing cash outflow in the consolidated statements of cash flows for the year ended December 31, 2023. No additional milestone payments were made or triggered in connection with this agreement during the three months ended March 31, 2024.

Acquisition of Worldwide Rights for TSHA-120 for the treatment of GAN

In March 2021, the Company acquired the exclusive worldwide rights to a clinical-stage AAV9 gene therapy program, now known as TSHA-120, for the treatment of GAN pursuant to a license agreement with Hannah's Hope Fund ("HHF") for Giant Axonal Neuropathy, Inc. TSHA-120 is an intrathecally dosed AAV9 gene therapy currently being evaluated in a clinical trial for the treatment of GAN.

Under the terms of the GAN Agreement, in exchange for granting the Company the exclusive worldwide rights to TSHA-120, HHF received an upfront payment of $5.5 million. No additional milestone payments were made or triggered in connection with the GAN Agreement during the three months ended March 31, 2024.

License Agreement for CLN7

In March 2022, the Company entered into a license agreement with UT Southwestern (the "CLN7 Agreement") pursuant to which the Company obtained an exclusive worldwide, royalty-bearing license with right to grant sublicenses to develop, manufacture, use, and commercialize licensed products for gene therapy for CLN7, a form of Batten Disease. In connection with the CLN7 Agreement, the Company paid a one-time upfront license fee of $0.3 million. The Company recorded the upfront license fee in research and development expense in the condensed consolidated statements of operations since the acquired license does not have an alternative future use. The Company is obligated to pay UT Southwestern up to $7.7 million in regulatory-related milestones and up to $7.5 million in sales-related milestones, as well as a low, single-digit royalty on net sales upon commercialization of the product. No additional milestone payments were made or triggered in connection with this agreement during the three months ended March 31, 2024.

Note 9—Stock-Based Compensation

On July 1, 2020, the Company's board of directors approved the 2020 Equity Incentive Plan ("Previous Plan") which permits the granting of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock awards ("RSAs"), restricted stock units ("RSUs") and other stock-based awards to employees, directors, officers and consultants. As of September 16, 2020, the approval date of the New Plan (as defined below), no additional awards will be granted under the Previous Plan. The terms of the Previous Plan will continue to govern the terms of outstanding equity awards that were granted prior to approval of the New Plan.

On September 16, 2020, the Company's stockholders approved the 2020 Stock Incentive Plan ("New Plan"), which became effective upon the execution of the underwriting agreement in connection with the IPO. The number of shares of common stock reserved for issuance under the New Plan automatically increases on January 1 of each year, for a period of ten years, from January 1, 2021, continuing through January 1, 2030, by 5% of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Company's board of directors. Pursuant to this provision, on January 1, 2024, the Company increased the number of shares of common stock reserved for issuance under the New Plan by 9,348,009 shares.

Furthermore, on September 16, 2020, the Company's stockholders approved the Employee Stock Purchase Plan ("ESPP"), which became effective upon the execution of the underwriting agreement in connection with the IPO. The maximum number of shares of common stock that may be issued under the ESPP will not exceed 362,000 shares of common stock, plus the number of shares of common stock that are automatically added on January 1st of each year for a period of up to ten years, commencing on the first January 1 following the IPO and ending on (and including) January 1, 2030, in an amount equal to the lesser of (i) one percent (1.0%) of the total number of shares of capital stock outstanding on December 31st of the preceding calendar year, and (ii) 724,000 shares of common stock. No shares were added to the ESPP in 2021. Pursuant to this provision, on January 1, 2023 and 2024,

the Company increased the number of shares of common stock reserved for issuance under the ESPP by 632,075 and 724,000 respectively. The Company has issued an aggregate of 188,193 shares of common stock under the ESPP as of March 31, 2024.

On December 15, 2023, the Company's board of directors adopted the Taysha Gene Therapies, Inc. 2023 Inducement Plan (the "Inducement Plan"). The Inducement Plan was adopted without stockholder approval pursuant to Nasdaq Listing Rule 5635(c)(4). The Board reserved 4,000,000 shares of the Company's common stock for issuance under the Inducement Plan.

The only persons eligible to receive grants of Inducement Awards (as defined below) under the Inducement Plan are individuals who satisfy the standards for inducement grants under Nasdaq Listing Rule 5635(c)(4). The Inducement Plan will be administered by the Board and the Company's Compensation Committee. Inducement Awards may only be granted by: (i) the Compensation Committee, provided such committee is comprised solely of "independent directors" (as defined by Nasdaq Listing Rule 5605(a)(2)) or (ii) a majority of the Company's "independent directors." An "Inducement Award" means any right to receive the Company's common stock, cash or other property granted under the Inducement Plan (including nonstatutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance stock awards, performance cash awards or other stock-based awards).

The number of shares available for grant under the Company's incentive plans were as follows:

	New Plan	Inducement Plan	Total
Available for grant - January 1, 2024	3,162,725	4,000,000	7,162,725
Plan adjustments and amendments	9,348,009	—	9,348,009
Grants	(11,314,622)	(784,700)	(12,099,322)
Forfeitures	120,227	—	120,227
Available for grant - March 31, 2024	1,316,339	3,215,300	4,531,639

Stock Options

For the three months ended March 31, 2024, 7,799,061 shares of common stock under the New Plan were awarded with a weighted-average grant date fair value per share of $1.35. The stock options vest over four years and have a ten-year contractual term.

The following weighted-average assumptions were used to estimate the fair value of time-based vesting stock options that were granted during the three months ended March 31, 2024 and 2023:

	Three Months Ended March 31,	
	2024	2023
Risk-free interest rate	3.95%	3.46%
Expected dividend yield	—	—
Expected term (in years)	6.1	6.1
Expected volatility	89%	81%

The following table summarizes time-based vesting stock option activity, during the three months ended March 31, 2024:

	Stock Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at January 1, 2024	5,960,922	$ 5.75	8.7	$ 1,960
Options granted	7,799,061	1.78		
Options cancelled or forfeited	(53,037)	2.65		
Options expired	(61,138)	17.79		
Outstanding at March 31, 2024	13,645,808	$ 3.44	9.2	$ 14,289
Options exercisable at March 31, 2024	1,948,590	$ 11.39	7.8	$ 763

The aggregate intrinsic value in the above table is calculated as the difference between the fair value of the Company's common stock at the respective reporting date and the exercise price of the stock options. As of March 31, 2024, the total unrecognized compensation related to unvested stock option awards granted was $16.9 million, which the Company expects to

recognize over a weighted-average period of approximately 2.8 years. No performance-based stock options were exercised during the period.

Performance Stock Options

In February 2023, the Company issued options to purchase 70,235 shares of common stock to employees under the New Plan that contain performance-based vesting conditions, subject to continued employment through each anniversary and achievement of the performance conditions. The grant date fair value of these awards was not material. As of March 31, 2024, 58,346 of the shares subject to the performance-based options were vested and outstanding. No stock options were exercised during the period.

In May 2023, the Company issued options to purchase 2,166,653 shares of common stock to employees under the New Plan that contain both service and performance-based vesting conditions (the "Original Options"), with a weighted average grant date fair value per share of $0.50. These Original Options were expected to vest over a 3.6 year term if a combination of clinical, regulatory and financing performance conditions were achieved. No compensation expense was recognized in 2023 related to the Original Options as achievement of the performance conditions was not considered probable. The following weighted-average assumptions were used to estimate the fair value of the options granted in February 2023 and the Original Options that were granted in May 2023:

Risk-free interest rate	4.02%
Expected dividend yield	—
Expected term (in years)	6.0
Expected volatility	81%

In December 2023, the Company modified all of the Original Options to amend the clinical and regulatory performance conditions and decreased the number of options granted to 1,516,655 (the “Modified Options”). The Company accounted for the changes in award terms as a modification in accordance with ASC 718, *Compensation - Stock Compensation*. Total compensation cost is equal to the modification date fair value. The Modified Options have a grant date fair value per share of $1.28. The following assumptions were used to estimate the fair value of the Modified Options:

Risk-free interest rate	3.90%
Expected dividend yield	—
Expected term (in years)	5.8
Expected volatility	88%

The Modified Options will vest over 3.0 years. The Company recognized stock-based compensation expense of $0.3 million for the three months ended March 31, 2024 related to the Modified Options. As of March 31, 2024, the total unrecognized compensation expense related to the Modified Options was $1.6 million, which the Company expects to recognize over a weighted average period of approximately 1.8 years using the accelerated attribution method. As of March 31, 2024, 1,516,655 of the Modified Options were outstanding. No Modified Options vested or were exercised during the period.

Market-based Stock Options

In February 2023, the Company issued options to purchase 70,233 shares of common stock to employees under the New Plan that contain a market-based vesting condition, subject to continued employment through each anniversary and achievement of the market condition. The grant date fair value of the stock options that contain market-based vesting conditions was not material. As of December 31, 2023, the market condition was not met and all 70,233 shares were forfeited.

Restricted Stock Units

For the three months ended March 31, 2024, the Company issued 4,300,261 RSUs to employees under the New Plan. The RSUs are subject to a service-based vesting condition. The service-based RSUs vest in equal annual installments over a four-year period. The Company at any time may accelerate the vesting of the RSUs. Such shares are not accounted for as outstanding until they vest.

The Company's default tax withholding method for RSUs granted prior to 2023 is the sell-to-cover method, in which shares with a market value equivalent to the tax withholding obligation are sold on behalf of the holder of the RSUs upon vesting and settlement to cover the tax withholding liability and the cash proceeds from such sales are remitted by the Company to taxing authorities. For RSUs granted in 2023, the Company's tax withholding policy allows the RSU holder to choose to either pay cash to the Company for the tax withholding obligation or elect the net withholding method, in which shares with a market equivalent to the tax withholding obligation are withheld and the net shares are issued to the RSU holder.

The Company's RSU activity for the three months ended March 31, 2024 was as follows:

	Number of Shares	Weighted Average Grant Date Fair Value per Share
Nonvested at January 1, 2024	375,044	$ 6.63
Restricted units granted	4,300,261	1.77
Vested	(17,334)	1.18
Cancelled or forfeited	—	—
Nonvested at March 31, 2024	4,657,971	$ 7.50

As of March 31, 2024, the total unrecognized compensation cost related to the unvested RSU's was $8.5 million which is expected to be amortized on a straight-line basis over a weighted-average period of approximately 3.2 years.

Performance and Market-based Restricted Stock Units

In February 2023, the Company issued 81,233 RSUs to employees under the New Plan that contain a combination of performance and market-based vesting conditions, subject to continued employment through each anniversary and achievement of market and performance conditions. The grant date fair value of the RSUs that contain performance and market-based vesting conditions was not material. As of December 31, 2023, 46,562 of the RSUs were forfeited and 34,671 RSUs vested and were settled. No RSUs that contain performance or market-based vesting conditions were granted or outstanding during the three months ended March 31, 2024.

Employee Stock Purchase Plan

In February 2022, the Company's board of directors authorized the first offering under the ESPP. Under the ESPP, eligible employees may purchase shares of Taysha common stock through payroll deductions at a price equal to 85% of the lower of the fair market values of the stock as of the beginning or the end of six-month offering periods. An employee's payroll deductions under the ESPP are limited to 15% of the employee's compensation and employees may not purchase more than 1,800 shares of Taysha common stock during any offering period. During the three months ended March 31, 2024 and 2023, stock-based compensation expense related to the ESPP was not material.

The following table summarizes the total stock-based compensation expense for the stock options, ESPP, RSAs and RSUs recorded in the condensed consolidated statements of operations for the three months ended March 31, 2024 and 2023 (in thousands):

	For the Three Months Ended March 31,	
	2024	2023
Research and development expense	$ 1,273	$ (263)
General and administrative expense	1,925	1,938
Total	$ 3,198	$ 1,675

Note 10—Warrants

Pre-Funded Warrants

On August 14, 2023, the Company entered into a Securities Purchase Agreement (the "August 2023 Purchase Agreement") with certain institutional and other accredited investors (the "Purchasers"), pursuant to which the Company agreed to sell and issue to the Purchasers in a private placement transaction (the "August 2023 Private Placement") (i) 122,412,376 shares (the "PIPE Shares") of the Company's common stock, and (ii) with respect to certain Purchasers, pre-funded warrants to purchase 44,250,978 shares of the Company's common stock (the "Pre-Funded Warrants") in lieu of shares of the Company's common stock. The purchase price per share of common stock was $0.90 per share (the "Purchase Price"), and the purchase price for the Pre-Funded Warrants was the Purchase Price minus $0.001 per Pre-Funded Warrant.

The Pre-Funded Warrants have a per share exercise price of $0.001, subject to proportional adjustments in the event of stock splits or combinations or similar events. The Pre-Funded Warrants will not expire until exercised in full. The Pre-Funded Warrants may not be exercised if the aggregate number of shares of common stock beneficially owned by the holder thereof immediately following such exercise would exceed a specified beneficial ownership limitation; provided, however, that a holder may increase or decrease the beneficial ownership limitation by giving 61 days' notice to the Company, but not to any percentage in excess of 19.99%. The exercise of the Pre-Funded Warrants was also contingent upon receipt of stockholder approval of an increase in the authorized shares of the Company's common stock (the "Stockholder Approval"), which the Company obtained at a special meeting of stockholders held on November 15, 2023.

The closing of the August 2023 Private Placement occurred on August 16, 2023 (the "Closing"). The total gross proceeds to the Company at the Closing were $150.0 million, and after deducting placement agent commissions and offering expenses payable by the Company, net proceeds were $140.3 million. The Company used the with-and-without method to allocate the total gross proceeds by first allocating the portion of the proceeds equal to the fair value of the Pre-Funded Warrants on the Closing date with the remaining proceeds allocated to the PIPE Shares on a residual basis.

The Company concluded that at the closing of the Private Placement in August 2023, the Pre-Funded Warrants did not meet the criteria for equity classification under the guidance of ASC 815 as the Company did not have sufficient authorized and unissued shares to satisfy the warrants if exercised. The Company recorded the Pre-Funded Warrants as liabilities at their fair value. This liability is subject to remeasurement at each balance sheet date and any change in fair value is recognized in the Company's consolidated statements of operations. The Company incurred $9.7 million of placement agent commissions and other issuance costs in connection with the August 2023 Private Placement. The placement agent commissions and other issuance costs were allocated between the PIPE Shares and the Pre-Funded Warrants on a systematic basis. The Company allocated $7.1 million to the PIPE Shares which was recorded as a deduction to additional paid-in capital. The remaining $2.6 million allocated to the Pre-Funded Warrants were recorded within general and administrative expense in the consolidated statements of operations for the year ended December 31, 2023. The issuance costs allocated to the Pre-Funded Warrants have been added back to net loss when deriving cash flows used in operations, and have been classified as a financing cash outflow in the consolidated statements of cash flows for the year ended December 31, 2023.

The Company measured the fair value of the PIPE Shares and Pre-Funded Warrants based on the $0.90 per share Purchase Price. The Company used the relative fair value method to allocate the net proceeds received from the sales of the PIPE Shares and the Pre-Funded Warrants on the consolidated balance sheet as follows (in thousands):

	Purchase Price Allocation	
PIPE Shares	$	110,127
Pre-Funded Warrants		39,826
Total	$	149,953

The Company remeasured the fair value of the Pre-Funded Warrants using the closing price of the Company's common stock on the Nasdaq Global Market as of November 15, 2023 of $1.68 per common share upon receipt of Stockholder Approval. The Company recorded a fair value adjustment of $34.5 million in the consolidated statements of operations for the year ended December 31, 2023 and the warrant liability of $74.3 million was reclassified into equity as an increase to additional paid-in capital upon receipt of Stockholder Approval.

SSI Warrants

In April 2023, the Company entered into a securities purchase agreement (the "SSI Securities Purchase Agreement"), with two affiliates of SSI Strategy Holdings LLC ("SSI"), named therein (the "SSI Investors") pursuant to which the Company agreed to issue and sell to the SSI Investors in a private placement (the "SSI Private Placement"), 705,218 shares of its common stock (the "SSI Shares") and warrants (the "SSI Warrants") to purchase an aggregate of 525,000 shares of the Company's common stock (the "Warrant Shares"). SSI provides certain consulting services to the Company. Each SSI Warrant has an exercise price of $0.7090 per

Warrant Share, which was the closing price of the Company's common stock on the Nasdaq Global Market on April 4, 2023. The SSI Warrants issued in the SSI Private Placement provide that the holder of the SSI Warrants will not have the right to exercise any portion of its SSI Warrants until the achievement of certain clinical and regulatory milestones related to the Company's clinical programs. The SSI Private Placement closed on April 5, 2023. Gross proceeds of the SSI Private Placement were $0.5 million.

The Company concluded that the SSI Warrants do not meet the criteria for equity classification under the guidance of ASC 815 due to settlement provisions that permit the holder to receive a variable number of shares in the event of a specified fundamental transaction as well as provisions that permit the holder to participate in dividends. As the SSI Warrants do not meet the criteria for equity classification, the Company recorded the warrants as liabilities at their fair value. This liability is subject to remeasurement at each balance sheet date until the warrants are exercised or expire, and any change in fair value is recognized in the Company's condensed consolidated statements of operations.

The Company determined the fair value of the SSI Warrants at issuance was $0.3 million using the Black-Scholes-Merton option pricing model. The following assumptions were used to estimate the fair value of the warrants at issuance:

Risk-free interest rate		3.46%
Expected dividend yield		—
Expected term (in years)		5.2
Expected volatility		81%
Market value of common stock (per share)	$	0.71

The fair value adjustment as of March 31, 2024 was $0.3 million using the Black-Scholes-Merton option pricing model. As of March 31, 2024, 316,667 of the SSI Warrants have vested and are exercisable. No warrants were exercised during the period.

The Company estimated the fair value of the SSI Warrant liability using the following assumptions as of March 31, 2024:

Risk-free interest rate		4.21%
Expected dividend yield		—
Expected term (in years)		4.5
Expected volatility		90%
Market value of common stock (per share)	$	2.87

The following table summarizes changes in the Company's warrant liability during the year ended March 31, 2024 (in thousands):

		Warrant Liability
Balance at January 1, 2024	$	454
Change in fair value		337
Balance at March 31, 2024	$	791

Note 11—Net Loss Per Common Share

Basic net loss per common share is computed by dividing net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding for the period. Since the Company had a net loss in all periods presented, basic and diluted net loss per common share are the same.

In August 2023, the Company issued liability-classified Pre-Funded Warrants with a nominal exercise price of $0.001 per share (see Note 10). In accordance with ASC 260, Earnings Per Share (ASC 260), shares issuable for little to no cash consideration should be included in the number of outstanding shares used to calculate basic loss per share as long as all conditions necessary for exercise are met. The conditions for exercise were met on November 15, 2023, at which time the Pre-Funded Warrants were reclassified into equity. The Pre- Funded Warrants are therefore included as outstanding shares as of November 15, 2023 to calculate the weighted average number of shares outstanding to calculate basic loss per share.

The following table represents the calculation of basic and diluted net loss per common share (in thousands, except share and per share data):

	For the Three Months Ended March 31,	
	2024	2023
Net loss	$ (24,061)	$ (17,622)
Weighted-average shares of common stock outstanding used to compute net loss per common share, basic and diluted	231,249,344	63,260,905
Net loss per common share, basic and diluted	$ (0.10)	$ (0.28)

The following common stock equivalents outstanding as of March 31, 2024 and 2023 were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been anti-dilutive:

	March 31, 2024	March 31, 2023
Unvested RSUs	4,657,971	750,081
Stock options	15,162,463	5,429,552
SSI Warrants	316,667	—
Total	20,137,101	6,179,633

Note 12—Income Taxes

Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. There is no provision for income taxes because the Company has incurred operating losses and capitalized certain items for income tax purposes since its inception and maintains a full valuation allowance against its net deferred tax assets. The reported amount of income tax expense for the period differs from the amount that would result from applying the federal statutory tax rate to net loss before taxes primarily because of the change in valuation allowance.

As of March 31, 2024, there were no material changes to either the nature or the amounts of the uncertain tax positions previously determined for the year ended December 31, 2023.

Note 13—Commitments and Contingencies

Litigation

From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities. The Company records a liability when a particular contingency is probable and estimable.

In January 2024 and April 2024, the Company was named a nominal defendant in two putative stockholder derivative actions filed by stockholders of the Company in the Court of Chancery of the State of Delaware. Shortly after filing suit, the plaintiff in the second-filed action voluntarily dismissed his lawsuit and filed a substantially similar action except with fewer named defendants. The complaints assert claims relating to the Company's August 2023 Private Placement against (i) certain of the Company's current and former directors and officers for breach of fiduciary duty and unjust enrichment; and (ii) against certain participants in the Company's August 2023 Private Placement for aiding and abetting breach of fiduciary duty and unjust enrichment. The complaints seek an unspecified award of damages in the Company's favor, plus pre-judgment and post-judgement interest, and an award to the plaintiffs for the costs and disbursement of the action, including fees for their attorneys, experts, and accountants. The Company has not recorded a liability related to these lawsuits because, at this time, the Company is unable to reasonably estimate possible losses or gains or determine whether an unfavorable outcome is either probable or remote.

Commitments

In the normal course of business, the Company enters into contracts that contain a variety of indemnifications with its directors, officers, employees, licensors, suppliers and service providers. The Company's maximum exposure under these arrangements is unknown at March 31, 2024. The Company does not anticipate recognizing any significant losses relating to these arrangements.

Note 14 – Strategic Reprioritization

In March 2022, the Company implemented changes to the Company's organizational structure as well as a broader operational cost reduction plan to enable the Company to focus on specific clinical-stage programs for GAN and Rett syndrome. Substantially all other research and development activities have been paused to increase operational efficiency.

In connection with prioritization of programs, the Company reduced headcount by approximately 35% across all functions in March 2022. In accordance with ASC 420, *Exit and Disposal Activities,* the Company recorded one-time severance and termination-related costs of $2.6 million in the condensed consolidated statements of operations for the three months ended March 31, 2022, primarily within research and development expenses. In December 2022 and throughout the first quarter of 2023, the Company further reduced headcount and recorded additional one-time severance and termination related costs of $2.7 million within research and development and general and administrative expenses.

Payment of these costs are substantially complete as of March 31, 2024. The amount of accrued severance recorded as of March 31, 2024 is as follows (amounts in thousands):

	Accrued Severance
Accrued severance as of January 1, 2024	$ 390
Severance recorded	—
Severance paid	(363)
Accrued severance as of March 31, 2024	$ 27

Note 15 – Retirement Plan

In July 2021, the Company adopted a 401(k) retirement savings plan that provides retirement benefits to all full-time employees. Eligible employees may contribute a percentage of their annual compensation, subject to Internal Revenue Service limitations. The Company contributed $0.2 million and $0.1 million to the 401(k) retirement savings plan for the three months ended March 31, 2024 and 2023, respectively.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q and the audited financial statements and notes thereto as of and for the year ended December 31, 2023 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, included in our Annual Report on Form 10-K for the year ended December 31, 2023, or Annual Report, filed with the Securities and Exchange Commission, or the SEC, on March 19, 2024. Unless the context requires otherwise, references in this Quarterly Report on Form 10-Q to "we," "us," and "our" refer to Taysha Gene Therapies, Inc. together with its consolidated subsidiaries.

Forward-Looking Statements

The information in this discussion contains forward-looking statements and information within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which are subject to the "safe harbor" created by those sections. These forward-looking statements include, but are not limited to, statements concerning our strategy, future operations, future financial position, future revenues, projected costs, prospects and plans and objectives of management. The words "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions, or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. These forward-looking statements involve risks and uncertainties that could cause our actual results to differ materially from those in the forward-looking statements, including, without limitation, the risks set forth in Part I, Item 1A, "Risk Factors" in our Annual Report. The forward-looking statements are applicable only as of the date on which they are made, and we do not assume any obligation to update any forward-looking statements.

Note Regarding Trademarks

All brand names or trademarks appearing in this report are the property of their respective holders. Unless the context requires otherwise, references in this report to the "Company," "we," "us," and "our" refer to Taysha Gene Therapies, Inc.

Overview

We are a clinical-stage biotechnology company focused on advancing AAV-based gene therapies for the treatment of severe monogenic diseases of the central nervous system, or CNS. Our lead clinical program TSHA-102 is in development for the treatment of Rett syndrome, a rare neurodevelopmental disorder with no approved disease-modifying therapies that address the genetic root cause of the disease. With a singular focus on developing transformative medicines, we aim to address severe unmet medical needs and dramatically improve the lives of patients and their caregivers. Our management team has proven experience in gene therapy development and commercialization. We leverage this experience, our manufacturing process and a clinically and commercially proven AAV9 capsid in an effort to rapidly translate treatments from bench to bedside.

We are evaluating TSHA-102 in the REVEAL Phase 1/2 adolescent and adult trial, which is a first-in-human, open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adolescent and adult females aged 12 years and older with Rett syndrome due to MECP2 loss-of-function mutation. The trial is taking place in Canada and the United States. We dosed the first two adult patients with Rett syndrome in 2023. There have been no treatment-emergent serious adverse events as of the 35-week assessment post-treatment for the first adult patient treated. In addition, there have been no treatment-emergent serious adverse events as of the 19-week assessment post-treatment for the second adult patient treated. The independent data monitoring committee, or IDMC, meeting to review the clinical data from the first two adult patients and the first pediatric patient took place in February 2024. The IDMC approved our request to proceed to earlier dose escalation in the adolescent and adult trial, enabling early advancement to cohort 2. The first patient in cohort 2 (high dose, $1x10^{15}$ total vg) has been enrolled, and dosing has been scheduled for the second quarter of 2024. We expect to provide an update on available safety and efficacy data from completed cohort 1 (low dose, $5.7x10^{14}$ total vg) in mid-2024. We expect to report initial available safety and efficacy data from cohort 2 (high dose, $1x10^{15}$ total vg) in the second half of 2024.

We are also evaluating TSHA-102 in the REVEAL Phase 1/2 pediatric trial, which is an open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in pediatric females with Rett syndrome due to MECP2 loss-of-function mutation. The trial is taking place in the United States. We submitted a CTA to the United Kingdom's Medicines and Healthcare Products Regulatory Agency, or MHRA, for pediatric patients with Rett syndrome and submitted an IND application for pediatric patients with Rett syndrome to the FDA for TSHA-102 early in the third quarter of 2023.

In August 2023, we received clearance from the FDA on our IND for TSHA-102 in pediatric patients with Rett syndrome and dosed the first Rett syndrome pediatric patient in December 2023. In February 2024, the IDMC provided clearance to dose the second pediatric patient following review of initial clinical data from the six-week post-treatment assessment from the first pediatric patient dosed. We dosed the second patient in cohort 1 in the first quarter of 2024. We expect to report initial available safety and efficacy data from cohort 1 (low dose, $5.7x10^{14}$ total vg) in mid-2024. We expect to report initial available safety and efficacy data from cohort 2 (high dose, $1x10^{15}$ total vg) in the second half of 2024.

We have received orphan drug designation and rare pediatric disease designation from the FDA and orphan drug designation from the European Commission for TSHA-102 for the treatment of Rett syndrome. We also received Fast Track Designation from the FDA for TSHA-102 for the treatment of Rett syndrome. We also received CTA clearance from the MHRA in early 2024. In February 2024, we received Innovative Licensing and Access Pathway, or ILAP, designation for TSHA-102 from the U.K. MHRA. The ILAP aims to facilitate patient access to novel treatments by accelerating time to market through opportunities for enhanced engagements with U.K. regulatory authorities and other stakeholders. In April 2024, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation for TSHA-102 in Rett syndrome. RMAT designation follows the FDA's review of available safety and efficacy data from the first three patients with Rett syndrome dosed with the low dose of TSHA-102 ($5.7x10^{14}$ total vg) across the REVEAL Phase 1/2 adolescent and adult trial and the REVEAL Phase 1/2 pediatric trial. RMAT designation was designed to expedite the development and review of regenerative medicine therapies. A regenerative medicine therapy is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious condition, and preliminary clinical evidence indicates the therapy has the potential to address unmet medical needs for such condition. Sponsor companies receiving RMAT designation can benefit from increased interactions with the FDA involving senior managers, with the goal of expediting drug development.

We have a limited operating history. Since our inception, our operations have focused on organizing and staffing our company, business planning, raising capital and entering into collaboration agreements for conducting preclinical and clinical development activities for our product candidates. Our lead product candidate is still in the clinical stage. We do not have any product candidates approved for sale and have not generated any revenue from product sales. Through March 31, 2024, we have funded our operations primarily through: (i) the sale of equity, raising an aggregate of $589.0 million of gross proceeds from our initial public offering, or the IPO, sales of common stock pursuant to our Sales Agreement (as defined below), our October 2022 follow-on offering and our 2023 private placements; (ii) pre-IPO private placements of our convertible preferred stock; (iii) our Term Loan Agreement (as defined below) and subsequently the Trinity Term Loan Agreement (as defined below); and (iv) the Astellas Transactions (as defined below).

On November 13, 2023, or the Trinity Closing Date, we entered into a Loan and Security Agreement, or the Trinity Term Loan Agreement, by and among us, the lenders party thereto from time to time, or the Trinity Lenders, and Trinity Capital Inc., as administrative agent and collateral agent for the Trinity Lenders, or Trinity. The Trinity Term Loan Agreement provides for, on the Trinity Closing Date, $40.0 million aggregate principal amount of term loans, or, collectively, the Trinity Term Loans. We drew the Trinity Term Loans in full on the Trinity Closing Date. The proceeds of the Trinity Term Loans were used to repay our obligations under the Loan and Security Agreement, or the Term Loan Agreement, with the lenders party thereto from time to time, or the Lenders and Silicon Valley Bank, as administrative agent and collateral agent for the Lenders, or the Agent, in full. The Term Loan Agreement with Silicon Valley Bank was terminated concurrently with entry into the Trinity Term Loan Agreement.

Since our inception, we have incurred significant operating losses. Our net losses were $24.1 million for the three months ended March 31, 2024 and $17.6 million for the three months ended March 31, 2023. As of March 31, 2024, we had an accumulated deficit of $537.1 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- continue to advance the clinical development of our product candidates and, if we determine to do so in the future, reprioritize the advancement of our preclinical and discovery programs;
- conduct our ongoing clinical trials of TSHA-102 and any other future product candidates that we advance;
- seek regulatory approval for any product candidates that successfully complete clinical trials;
- continue to develop our gene therapy product candidate pipeline;
- scale up our clinical and regulatory capabilities;
- work with CMOs to manufacture current Good Manufacturing Practice, or GMP material for clinical trials or potential commercial sales;
- establish a commercialization infrastructure and scale up internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;

- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing quality control, regulatory, manufacturing and scientific and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

Our Pipeline

We are focused on discovering, developing and commercializing gene therapies for the treatment of monogenic diseases of the CNS, in both rare and large patient populations. Our primary focus is advancing our lead TSHA-102 clinical program in Rett syndrome, while our pipeline of CNS programs offers the potential for additional development opportunities in the future. The stage of development of our Rett syndrome program, including the progress in our ongoing clinical trials, is represented in the below table:

As of the date of this report, we have deprioritized the company-sponsored evaluation of certain clinical-stage programs, including TSHA-120 for GAN, TSHA-105 for SLC13A5, TSHA-118 for CLN1 and TSHA-121 for CLN7, and are seeking external strategic options to potentially enable further development of these programs.

TSHA-102 for Rett Syndrome

TSHA-102 is a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare progressive neurodevelopmental disorder caused by mutations in the X-linked MECP2 gene encoding methyl CpG-binding protein 2, or MeCP2, which is essential for regulating neuronal and synaptic function in the brain. The disorder is characterized by loss of communication and hand function, slowing and/or regression of development, motor and respiratory impairment, seizures, intellectual disabilities and shortened life expectancy. Rett syndrome progression is divided into four key stages, beginning with early onset stagnation at 6 to 18 months of age followed by rapid regression, plateau and late motor deterioration. Rett syndrome primarily occurs in females and is one of the most common genetic causes of severe intellectual disability.

Designed as a one-time treatment, TSHA-102 aims to address the genetic root cause of the disease by delivering a functional form of MECP2 to cells in the CNS. The vector is delivered directly to the cerebrospinal fluid via intrathecal administration, which facilitates optimal biodistribution and cell transduction within key regions of the CNS. Because of the risks associated with both under- and over-expression of MeCP2, we have combined high-throughput microRNA, or miRNA, profiling and genome mining to create miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel. The miRARE element includes binding sites for endogenous miRNA, which are responsive to MeCP2 levels to prevent overexpression. By utilizing the miRARE technology, TSHA-102 is designed to mediate levels of MeCP2 in the CNS on a cell-by-cell basis without risk of overexpression. By increasing MECP2 levels in MECP2 deficient cells and maintaining healthy levels of MECP2 output of healthy cells, TSHA-102 has demonstrated the ability to produce and maintain safe transgene expression levels in the CNS. (Sinnet, SE, et al. Engineered microRNA-based regulatory element permits safe high-dose miniMECP2 gene therapy in Rett mice. Brain. 2021 awab182.)

Currently, there are no approved disease-modifying therapies that treat the genetic root cause of Rett syndrome, and there is a significant unmet medical need. According to the Rett Syndrome Research Trust, Rett syndrome affects more than 350,000 patients

worldwide. The estimated addressable patient population with typical Rett syndrome caused by a pathogenic/likely pathogenic MECP2 mutation is between 15,000 and 20,000 patients in the United States, European Union and United Kingdom.

Phase 1/2 REVEAL Clinical Trials

We currently have two Phase 1/2 clinical trials ongoing for TSHA-102: an adolescent/adult study in the United States and Canada and a pediatric study in the United States. In addition, approval has been granted to open a pediatric study in the U.K. The trials are described below:

Study	Primary Study Objectives	Study Population	TSHA-102 Single IT Dosing Regimen	Active Geographies
REVEAL Phase 1/2 Adolescent and Adult Trial	To assess the safety, tolerability, and preliminary efficacy of TSHA-102 as a single lumbar IT administration	Adolescent and adult females with Rett syndrome, ≥12 years old	Part A: Cohort 1: 5.7×10^{14} vg IT Cohort 2: 1.0×10^{15} vg IT Part B: Dose Expansion with MTD or MAD	Canada, US
REVEAL Phase 1/2 Pediatric Trial	To assess the safety, tolerability, and preliminary efficacy of TSHA-102 as a single lumbar IT administration	Pediatric females with Rett syndrome Part A: 5-8 years old Part B: 3-8 years old	Part A: Cohort 1: 5.7×10^{14} vg IT Cohort 2: 1.0×10^{15} vg IT Part B: Dose Expansion with MTD or MAD in two age cohorts	US, UK

IT – Intrathecal; MTD – Maximum Tolerated Dose; MAD – Maximum Administered Dose.

We dosed the first adult patient with Rett syndrome in May 2023. The second adult patient was dosed in September 2023. We dosed the first pediatric patient with Rett syndrome in the Phase 1/2 REVEAL pediatric trial in December 2023 and the second pediatric patient was dosed in the first quarter of 2024. In early 2024, we announced that the U.K. MHRA authorized the CTA for TSHA-102 in pediatric patients with Rett syndrome, enabling expansion of our ongoing pediatric trial into the United Kingdom. In February 2024, we announced the expansion of the ongoing REVEAL Phase 1/2 adolescent and adult trial in Canada into the United States following submission of the adolescent and adult trial protocol to the FDA.

Cohort 1 of both trials is evaluating the low dose of TSHA-102 of $5.7x10^{14}$ total vg. Two adult patients have been dosed in cohort 1 in the adolescent and adult trial, and TSHA-102 showed a well-tolerated safety profile with no treatment-emergent serious adverse events as of the week 35 post-treatment assessment for the first adult patient and as of the week 19 post-treatment assessment for the second adult patient. Following review of available clinical data from the first two dosed adult patients and first dosed pediatric patient showing that TSHA-102 was generally well-tolerated, and in light of the potential for improved benefit at the higher dose ($1x10^{15}$ total vg), in February 2024, the IDMC approved our request to proceed to earlier dose escalation in the adolescent and adult trial, enabling earlier advancement to cohort 2 evaluating $1x10^{15}$ total vg. Dosing in cohort 1 of the adolescent and adult trial is now considered complete. We have enrolled the first patient in cohort 2 of the adolescent and adult trial and scheduled dosing for the second quarter of 2024. The IDMC also approved the dosing of the second patient in cohort 1 of the pediatric trial, and the second pediatric patient was dosed in the first quarter of 2024.

We expect to report initial available safety and efficacy data from cohort 1 of the pediatric trial and an update on available safety and efficacy data from completed cohort 1 (low dose, $5.7x10^{14}$ total vg) of the adolescent and adult trial in mid-2024. We expect to report initial available safety and efficacy data from cohort 2 (high dose, $1x10^{15}$ total vg) of both the adolescent and adult trial and the pediatric trial in the second half of 2024.

The maximum tolerated dose or maximum administered dose established in Part A will be administered during dose expansion in Part B. Data from Part A will be assessed by regulatory agencies and the IDMC to determine key elements of Part B of the study, including efficacy endpoints, study duration and the MTD or MAD.

TSHA-102 REVEAL Adolescent / Adult Clinical Trial TSHA-102-CL-101 Safety and Efficacy Summary

Efficacy endpoints include patient assessments performed by clinicians using the Clinical Global Impressions Scale – Improvement, or CGI-I, the Clinical Global Impressions Scale – Severity, or CGI-S, Rett Syndrome Hand Function Scale, or RSHFS, Vineland Adaptive Behavior Scales Third Edition, and Revised Motor Behavior Assessment, or R-MBA. Additional efficacy endpoints also include patient assessments by caregivers using Parental Global Impressions Improvement, or PGI-I, the Rett Syndrome Behavior Questionnaire, or RSBQ, Seizure Diaries and other clinical assessment scales.

The first adult patient has the most advanced stage of Rett syndrome, Stage IV, with a genetic change consisting of a large deletion within the MECP2 gene that is known to cause Rett syndrome. This patient's phenotypic manifestation is severe, having lost all abilities to: walk, stand, and sit without support around age eight (non-ambulatory, wheelchair bound, limited movements of her lower extremities), use her hands around age six (unable to grasp and hold objects of any size) and speak around age two (non-verbal, minimal vocalizations). Per the Principal Investigator, or PI, the first adult patient’s baseline reported seizure frequency was approximately two to four seizures per year. After TSHA-102 administration, the first adult patient has showed a well-tolerated safety profile with no treatment-emergent serious adverse events as of the 35-week safety assessment post-treatment. Per the protocol, prophylactic immunosuppressant therapy began seven days prior to TSHA-102 administration. The first adult patient’s steroid taper was initiated on week 17 and was completed by week 33. At week 25, the patient demonstrated sustained and new improvements across key efficacy assessments at decreased steroid levels compared to earlier post-treatment assessments and the patient was subsequently at physiologic levels within a few days post the week 25 assessment.

As of 35 weeks post-treatment, following completion of the steroid taper, the PI noted that the first adult patient’s improvements observed across multiple clinical domains had been sustained with new improvements as well. These include sustained improvements in motor function, with the gained ability to kick legs against gravity and sit unassisted for the first time in over one decade, including after the steroids have been fully tapered from the patient at week 35 post-treatment, as supported by video evidence. Further sustained improvements from the first adult patient were also observed in motor function, including improved hand function as the patient’s hands were more open, and the improved ability to grasp and hold objects. The PI also observed new improvements in the patient’s socialization/communication skills at week 35 post-treatment, as the patient was more alert and interactive during the day, made more vocalizations, and showed the enhanced ability to use her eye-driven communication device, which caregivers reported she had not expressed interest in before treatment. Specifically, at week 35 post-treatment, the patient was able to use the eye-driven device much more efficiently, with the gained ability to activate functions on the screen of the device. Difficulty in communication, including loss of speech, is one of the most prominent symptoms of Rett syndrome and a key area of concern for caregivers as it interferes with patients’ ability to communicate their needs and express their interests. Further, the first adult patient showed sustained improvements in autonomic function at week 35 post-treatment, including sustained normalized sleep/night-time behaviors for the first time in twenty years, improved breathing patterns with fewer breath holding spells and infrequent hyperventilation compared to before treatment, and improved circulation with hands and feet at a more normal temperature and color. Finally, the PI observed that the patient’s seizures had been overall well controlled through week 35 following treatment at lower levels of anti-seizure medication, relative to baseline, and the patient no longer experienced unprovoked seizures. These observations are supported by data from the Seizure diaries. The PI’s clinical observations are supported by clinical and video evidence as well as caregiver-reported seizure diaries.

The second adult patient has the most advanced stage of Rett syndrome, Stage IV, with a missense mutation in the MECP2 gene, which has been reported in over 25 publications to cause Rett syndrome. This patient's phenotypic manifestation is milder than the first adult patient, with partial loss of ambulation (able to walk/stand without support, wide based, slow, unsteady gait) and hand function (with significant stereotypies that emerged by age three, inability to transfer objects between hands). She has been nonverbal since the age of two years old. Per the PI, the second adult patient’s baseline reported seizure frequency was approximately two to four seizures per week. After TSHA-102 administration, the patient has showed a well-tolerated safety profile with no treatment-emergent serious adverse events as of the 19-week safety assessment post-treatment. Tapering of the steroids was initiated on week 17 and is expected to be complete by week 25.

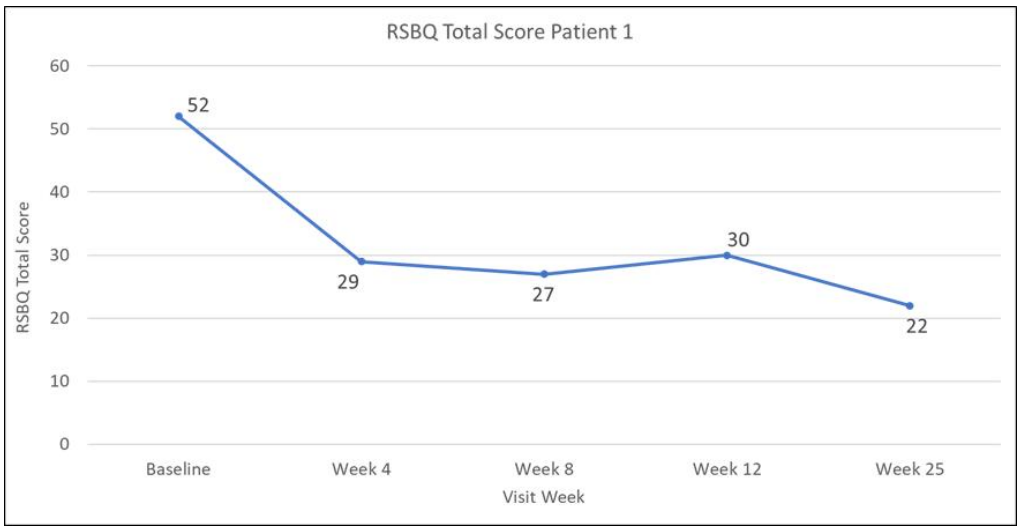
As of 19-weeks post-treatment, the PI noted that the second adult patient’s improvements observed across multiple clinical domains had been maintained with new improvements at decreased steroid levels relative to earlier post-treatment assessments. These include sustained improvement in motor function, with the patient’s hands more open and relaxed, and improved hand stereotypies, with less forceful hand wringing. These observations from the PI are supported by video evidence. The second adult patient also showed sustained improvements in social/communication skills as she was more interested, engaged and alert, including showing increased response to spoken words and eye contact, as supported by improved social skills on the clinician administered R-MBA scale. The second adult patient also showed sustained improvements in autonomic function, including improved breathing patterns with fewer breath holding spells and infrequent hyperventilation compared to before treatment, and improved circulation, with hands and feet at a more normal temperature and color. Finally, the second adult patient showed pronounced improvements in seizure

frequency at week 19 post-treatment, with a significant reduction in seizures at 25% lower levels of anti-seizure medication, relative to baseline. The PI noted that the patient's epilepsy had been much better controlled following treatment at a lower dose of anti-seizure medication, and has been seizure-free for 17 weeks as of the week 19 post-treatment time point, despite a pre-treatment seizure frequency of approximately two to four seizures per week. The PI's clinical observations are supported by clinical and video evidence as well as caregiver-reported seizure diaries.

TSHA-102-CL-101 Trial Adult Patient 1 Efficacy Data

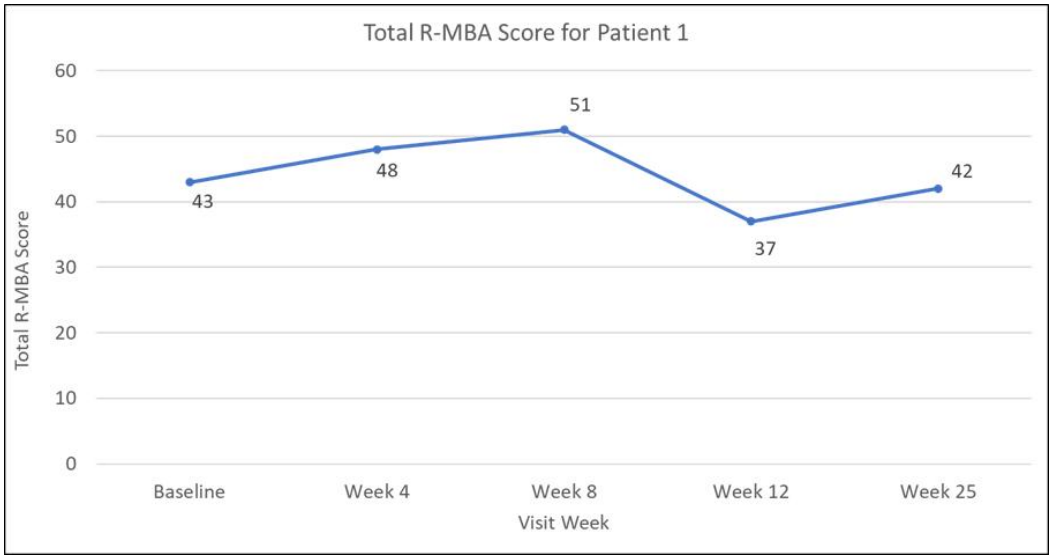
The first adult patient showed clinically significant improvement in CGI-S from score of six (severely ill) at baseline to score of five (markedly ill) in this measure four weeks post-TSHA-102 administration and this improvement was sustained through week 25. Similarly, the patient demonstrated sustained improvement in CGI-I and PGI-I as of week 25 assessment post-TSHA-102 administration with scores of two (much improved and much better for CGI-I and PGI-I, respectively).

The first adult patient dosed with TSHA-102 demonstrated a sustained clinical improvement in RSBQ Total Score at week 25 post-TSHA-102 administration as depicted in the chart below.



Compared to baseline, a 30-point improvement was observed in RSBQ total score at week 25 post TSHA-102 administration. RSBQ changes at week 25 were driven by improvements in hand behaviors, breathing problems, general mood, repetitive face movements, night-time behaviors, fear/anxiety, and body rocking/expressionless face.

As shown in the diagram below, the first adult patient started to show notable overall improvements in R-MBA total scores at the week-12 visit. However, the total R-MBA score increased closer to baseline at the week 25 visit where improvements were demonstrated in motor dysfunction and respiratory behaviors.



The first adult patient has demonstrated stable seizure events relative to baseline through week 35 post-treatment, based on caregiver-reported medical history, and seizures are confined to periods where phenytoin level declines to <50 µmol/L (previously <100 µmol/L). The first adult patient has been on phenytoin as antiepileptic therapy, which she has continued following treatment with TSHA-102. Prior to treatment and per medical history, the patient required phenytoin levels of >100 µmol/L to control her seizures. The first adult patient had seizures prior to TSHA-102 administration on Day –8 and Day –7, and post-administration she had seizures on Days 45-49 and Day 82 associated with lower than target phenytoin levels. Specifically, the seizures on Days 45-49 corresponded with a phenytoin level of 45.9 µmol/L, and the seizure on Day 82 corresponded with a phenytoin level was 35.9 umol/L.

Loss of hand function is a hallmark characteristic of Rett syndrome and a key area of concern for caregivers. It impacts a patient's ability to communicate and impedes daily activities, which ultimately limits independence. The RSHFS is a scale designed to evaluate hand function in patients with Rett syndrome. Hand function is evaluated by an experienced independent physical therapist with expertise in evaluating hand function in patients with Rett syndrome. Sessions are videotaped in which the patient's caregiver offers the patient both large (e.g. a toy, cup, or spoon) and small (e.g. a grape or small piece of sandwich) objects so that she may demonstrate her ability to grasp, pick up, and hold the objects. The independent physical therapist then codes the demonstrated hand function in each video at one of four levels of hand function, ranging from no active grasping of any objects to independent grasping, for the best level for large objects assessment.

The first adult patient showed an improvement in RSHFS at 25 weeks post-TSHA-102 administration as depicted in the tables below. As of week 25 following treatment, the first adult patient is using her non-dominant hand for some basic grasping whereas before treatment, she was not able to grasp at all. As of the week 25 assessment, her dominant hand function improved from

baseline with the demonstrated ability to grasp of two different objects (spoon and toy) rather than just one object (spoon). These clinical observations reported by the independent physical therapist are supported by video evidence.

Dominant Hand		
Visit	**Best Level for Large Object**	**Number of Objects Grasped**
Baseline	3	1
Week 8	2	2
Week 10	3	2
Week 11	3	2
Week 25	3	2

Non-Dominant Hand		
Visit	**Best Level for Large Object**	**Number of Objects Grasped**
Baseline	NA*	0
Week 8	1	0
Week 10	2	2
Week 11	3	2
Week 25	2	1

***RSHFS for patient 1's non-dominant hand was not assessed at baseline**

Best Level Scoring Criteria:

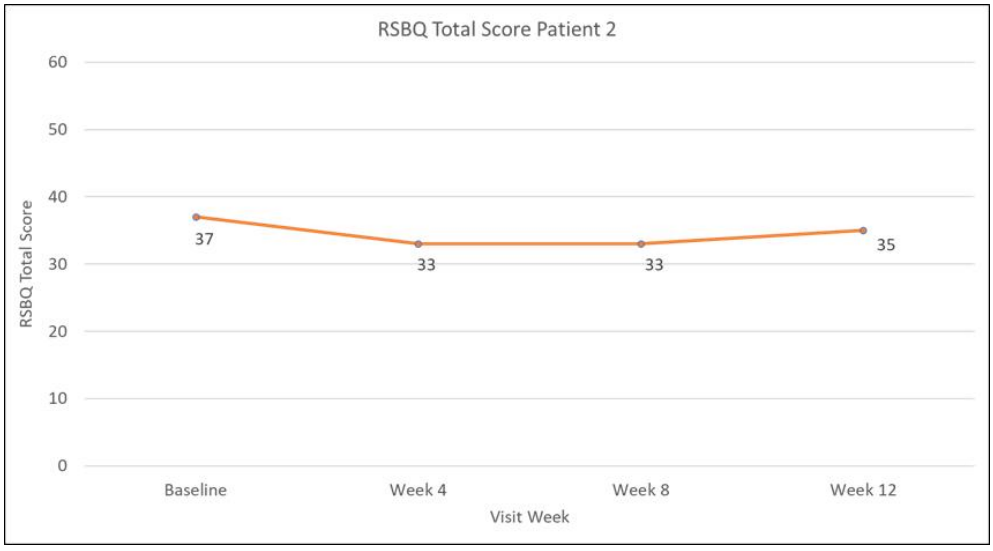
Level	Description
1	No Active Grasping
2	Assisted to Grasp (hold 2 seconds)
3	Hold (at least 2 seconds)
4	Independent Grasp (pick up and hold)

TSHA-102-CL-101 Trial Adult Patient 2 Efficacy Data

While the two adult patients dosed to date in our REVEAL trial both have the most advanced stage of Rett syndrome, Stage, IV, they possess different genetic backgrounds and mutation types, which manifest in different phenotypes and clinical severity.

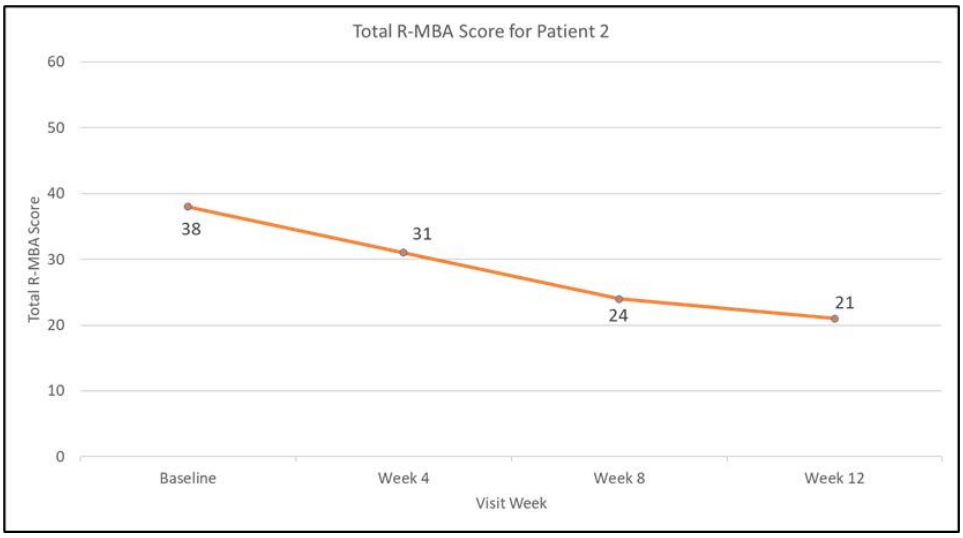
While there was no change at 12 weeks post TSHA-102 administration in the second adult patient's CGI-S score of four (moderately ill) at baseline, her CGI-I and PGI-I scores show sustained improvement (score of three, minimally better and a little better, respectively) at week 12.

The second adult patient showed a sustained clinical improvement in RSBQ Total Score 12 weeks post-TSHA-102 administration as depicted in the chart below.



Improvements were documented in several subdomains. Specifically, compared to baseline, improvements were noted in breathing, body rocking, facial expressions, general mood, and repetitive face movements.

The second adult patient showed continued improvement in the R-MBA Total Score at 12 weeks post-TSHA-102 administration as depicted in the graph below.



A 17-point improvement at week 12 was demonstrated in the R-MBA Total Score. Most notable improvements were documented in the following subscales: social skills, respiratory behaviors, seizures, truncal rocking and stereotypic hand movements and/or mouthing, and aberrant behaviors.

Seizure diary demonstrated reduced seizure events relative to baseline through 19-weeks post-treatment for the second adult patient at lower levels of anti-seizure medication, based on caregiver-reported medical history. Pre-treatment, the second adult patient had approximately two to four seizures per week, and there has been a significant reduction in seizures post-treatment with TSHA-102 at 25% lower levels of anti-seizure medication, relative to baseline. Post-treatment, the second adult patient had a single seizure event on day 13 as of week 19 post-treatment. The seizure was an unknown type, with motor manifestations and lasted less than one minute duration. The patient has been seizure-free for 17 weeks as of the week 19 post-treatment time point.

Hand function in the dominant hand for the second adult patient is challenging to interpret due to inconsistency in the video recording. At the week 8 post-treatment assessment, the second adult patient's dominant hand received a hand function score of four, an independent grasp (pick up and hold) and was able to grasp three objects. Hand function in the second adult patient remained unchanged in the non-dominant hand on the RSHFS 12 weeks post-TSHA-102 treatment. These clinical observations reported by the independent physical therapist are supported by video evidence.

Dominant Hand*		
Visit	**Best Level for Large Object**	**Number of Objects Grasped**
Baseline**	NE	0
Week 4**	NE	0
Week 8	4	3
Week 12**	NE	1

Non-Dominant Hand*		
Visit	**Best Level for Large Object**	**Number of Objects Grasped**
Baseline	1	0
Week 4	1	0
Week 8	1	0
Week 12	1	0

***Rater Note:** *Participant 2 does initiate grasping, more with right hand, but grasp is extremely weak and not functional*

**Video evaluation not evaluable at baseline, week 4 and week 12 - due to inadequate initial caregiver training and consistency by caregiver when recording video assessment, the assessment was not conducted as defined in the guidelines.

Best Level Scoring Criteria:

Level	Description
1	No Active Grasping
2	Assisted to Grasp (hold 2 seconds)
3	Hold (at least 2 seconds)
4	Independent Grasp (pick up and hold)
NE	Not Evaluable

Deprioritized Programs

We have previously deprioritized the evaluation of our clinical product candidates TSHA-120 for GAN, TSHA-105 for SLC13A5, TSHA-118 for CLN1 and TSHA-121 for CLN7. Although we are not currently evaluating the potential of TSHA-105, TSHA-118 and TSHA-121, we may again evaluate any of these in the future as a product candidate as a component of our pipeline expansion plans, or pursue partnerships to advance these programs.

TSHA-120 for Giant Axonal Neuropathy (GAN)

GAN is an ultra-rare autosomal recessive, progressive neurodegenerative disease of the central, peripheral and autonomic nervous systems caused by deficiency or complete loss-of-function of gigaxonin and the accumulation of intermediate filaments. Epidemiology studies indicate there are between 1,000 and 1,500 treatable GAN patients in the United States, European Union and United Kingdom.

There is an early (classical) and late-onset (non-classical) phenotype associated with the disease, with shared pathophysiology due to accumulation of intermediate filaments. Symptoms and features of children with classical GAN usually develop before the age of five years with distal muscle weakness and sensory loss due to axonal sensory motor neuropathy, manifesting as bilateral foot drop and difficulties with fine motor coordination. An abnormal, wide based, unsteady gait due to CNS and cerebellar involvement is also a common initial clinical manifestation. Children with the classical phenotype typically have dull, tightly curled, coarse hair ("kinky" hair), "giant" axons pathognomonic on a nerve biopsy due to accumulation of intermediate filaments, and progressive spinal cord atrophy and white matter abnormalities, initially around the cerebellar dentate nucleus, on MRI images. Symptoms progress and, as the children grow older, they develop progressive proximal muscle weakness, resulting in difficulties raising their arms and standing from the floor or a chair, scoliosis, distal contractures, progressive gait and limb ataxia, leading to loss of ambulation by the second decade. Progressive optic nerve atrophy, seen early in the disease, results in increasing deterioration of visual acuity in later stages and has been more recently described. Indeed, decreased visual acuity was seen at baseline in approximately half of GAN patients aged 3-21 years, enrolled in a natural history study [Brain. 2021 Nov 29;144(10):3239-3250]. Due to increased respiratory muscle weakness and restrictive respiratory failure as a result of severe scoliosis, assisted ventilation is required in adolescents. GAN patients often die during their late teens or early twenties, typically due to respiratory failure.

The late-onset, or non-classical, phenotype is often categorized as Charcot-Marie-Tooth Type 2, or CMT2, as it presents as a typical early onset axonal sensory motor neuropathy without the typical kinky hair and CNS involvement of the classical phenotype and has a relatively slow progression. This phenotype might represent up to 6% of all CMT2 diagnosis. In the late-onset population, patients have poor quality of life and significantly compromised activities of daily living. The disease is life limiting but not as severely as classic GAN. In classic GAN, symptomatic treatments attempt to maximize physical development and minimize the rate of deterioration. Currently, there are no approved disease-modifying therapies available, only palliative treatments.

In March 2021, we acquired the exclusive worldwide rights to a clinical-stage, intrathecally dosed AAV9 gene therapy program, now known as TSHA-120, for the treatment of GAN, pursuant to a license agreement with Hannah's Hope Fund for Giant Axonal Neuropathy, Inc., or HHF. Under the terms of the agreement, HHF received an upfront payment of $5.5 million and will be eligible to receive clinical, regulatory and commercial milestones totaling up to $19.3 million, as well as a low, single-digit royalty on net sales upon commercialization of TSHA-120. We received orphan drug designation and rare pediatric disease designation from the FDA for TSHA-120 for the treatment of GAN. In April 2022, we received orphan drug designation from the European Commission for TSHA-120 for the treatment of GAN.

In September 2023, subsequent to the receipt of Type C meeting feedback from the FDA regarding a registrational path for TSHA-120, we announced that we discontinued the development of our TSHA-120 program in the evaluation for the treatment of GAN. In January 2024, we initiated the transfer of the FDA IND application and investigational clinical trial material for TSHA-120 in GAN to clinical trial collaborator NINDS, creating an opportunity for continued clinical evaluation of TSHA-120 in GAN. Additionally, we have entered discussions with the originating advocacy organization regarding TSHA-120 in an effort to transfer rights back to the advocacy organization to move the program forward.

TSHA-118 for CLN1 Disease

CLN1 disease (one of the forms of Batten disease), a lysosomal storage disorder, is a progressive, fatal neurodegenerative disease with early childhood onset that has an estimated incidence of approximately 1 in 138,000 live births worldwide. The estimated prevalence of CLN1 disease is 1,000 patients in the United States and European Union. CLN1 disease is caused by loss-of-function mutations in the CLN1 gene that encodes the enzyme palmitoyl-protein thioesterase-1, a small glycoprotein involved in the

degradation of certain lipid-modified proteins. Loss of function mutations in the CLN1 gene causes accumulation of these lipid-modified proteins in cells, eventually leading to aggregation, neuronal cellular dysfunction and ultimately neuronal cell death.

In the infantile-onset form of CLN1 disease, clinical symptoms appear between six to 24 months and include rapid deterioration of speech and motor function, refractory epilepsy, ataxia and visual failure. Infantile-onset CLN1 patients are typically poorly responsive by five years of age and remain noncommunicative until their death, which usually occurs by seven years of age. Late-infantile-onset CLN1 disease begins between two to four years of age with initial visual and cognitive decline followed by the development of ataxia and myoclonus, or quick, involuntary muscle jerks. Juvenile-onset CLN1 disease patients present between the ages of five to ten years old, with vision loss as a first symptom followed by cognitive decline, seizures and motor decline. Approximately 60% of the children diagnosed with CLN1 disease in the United States present with early-onset infantile forms, with the remaining 40% experiencing later-onset childhood forms.

All currently available therapeutic approaches for patients with CLN1 disease are targeted towards the treatment of symptoms, and no disease-modifying therapies have been approved. Gene therapy has shown promise in correcting forms of neuronal ceroid lipofuscinoses diseases that involve mutations in soluble enzymes, in part, due to cross-correction of neighboring non-transduced cells.

We believe that the introduction of a functional *CLN1* gene using an AAV9 vector delivered intrathecally to the CNS offers the potential of a disease-modifying therapeutic approach for this disease. TSHA-118 is a self-complementary AAV9 viral vector that expresses human codon-optimized CLN1 complementary deoxyribonucleic acid under control of the chicken ß-actin hybrid promoter. We acquired exclusive worldwide rights to certain intellectual property rights and know-how relating to the research, development and manufacture of TSHA-118 (formerly ABO-202) in August 2020 pursuant to a license agreement with Abeona Therapeutics Inc., or Abeona.

TSHA-118 has been granted orphan drug designation, rare pediatric disease designation and fast track designation from the FDA and orphan drug designation from the European Medicines Agency for the treatment of CLN1 disease.

There is currently an open IND for the CLN1 program. We submitted a CTA filing for TSHA-118 which was approved by Health Canada in 2021. Clinical trial material has been manufactured and released and is now ready for use in a clinical trial setting. We provided investigational clinical trial material for TSHA-118 in CLN1 to support an individual-patient investigator-initiated IND request from RUSH University Medical Center for the treatment of a patient with CLN1 disease.

TSHA-105 for SLC13A5 Deficiency

TSHA-105 is a gene replacement therapy in development for the treatment of SLC13A5 deficiency, a rare autosomal recessive epileptic encephalopathy characterized by the onset of seizures within the first few days of life. SLC13A5 deficiency is caused by bi-allelic loss-of function mutations in the SLC13A5 gene, which codes for a sodium dependent citrate transporter, or NaCT, that is largely expressed in the brain and liver. To date, all tested mutations result in no or a greatly reduced amount of the citrate in the cells. Diminished NaCT function leads to loss of neuronal uptake of citrate and other metabolites such as succinate that are critical to brain energy metabolism and function. Affected children have impairments in gross motor function and speech production with relative preservation of fine motor skills and receptive speech. Currently, there are no approved therapies for SLC13A5 deficiency, and treatment is largely to address symptoms. The estimated prevalence of SLC13A5 deficiency is 1,900 patients in the United States and European Union.

TSHA-105 is constructed from a codon-optimized human SLC13A5 gene packaged in a self-complementary AAV9 capsid.

We have received orphan drug designation and rare pediatric disease designation from the FDA and orphan drug designation from the European Commission for TSHA-105 for the treatment of epilepsy caused by SLC13A5 deficiency. Clinical trial material has been manufactured and released and is now ready for use in a clinical trial setting.

Other Programs

We have a pipeline of early-stage gene therapy programs targeting CNS diseases that we may progress in the future or advance through potential partnerships.

TSHA-113 for Tauopathies

We are developing TSHA-113 for the treatment of tauopathies. Tauopathies comprise a large subset of neurodegenerative diseases involving the aggregation of microtubule associated protein tau, or MAPT, protein into neurofibrillary or gliofibrillary tangles in the human brain. These include MAPT-associated frontotemporal dementia, or FTD, progressive supranuclear palsy, or PSP,

corticobasal degeneration, or CD, and Alzheimer's disease. There are an estimated 11,000 patients in United States and Europe affected by MAPT mediated FTD and 2,000 to 2,500 are affected with MAPT-mediated PSP. and CD, and Alzheimer's disease affects an estimated 6.2 million Americans and 7.8 million Europeans.

Intrathecal delivery of an antisense oligonucleotide, or ASO, targeting Tau mRNA by Biogen/Ionis in a Phase 1 study demonstrated durable, robust, time and dose dependent lowering of tau protein and phospho-tau in cerebrospinal fluid of Alzheimer's disease patients. Buoyed by these results, in August 2022, Biogen started a Phase 2 trial in people with mild cognitive impairment or mild dementia due to Alzheimer's disease. This ASO target validation paved the way for other approaches targeting intercellular tau mRNA (reduce tau protein production), for treating Tauopathies.

Unlike an ASO treatment, which would require repeat lifelong administration, we are developing a one-time treatment for Tauopathies. TSHA-113 is an AAV9 capsid that packages a tau-specific miRNA and is delivered in the cerebrospinal fluid for the treatment of tauopathies. This miRNA targets all six isoforms of tau mRNA.

We tested the efficacy of TSHA-113 in PS19 mice, a validated mouse model for tauopathies. These mice express human MAPT, and they exhibit significant tau pathology, neurodegeneration, loss of body weight and progressive hind-limb paralysis around nine to 12 months of age. We tested efficacy of our treatment by delivering TSHA-113 to PS19 mice at three months, six months and nine months of age via intracisterna magna injection. We found that the tau mRNA and protein levels were significantly reduced by TSHA-113 treatment. Consistently, the tau seeding assay showed reduced levels of pathological tau in brains from PS19 mice treated with TSHA-113. In addition, TSHA-113 treatment was able to rescue the survival rate, loss in body weight, and the hind limb clasping phenotype in the PS19 mice when treated at three months, six months and nine months of age. Taken together, these results demonstrate that a one-time, vectorized delivery of a tau-specific miRNA is a promising approach for treatment for tauopathies. Ongoing and future work is focused on optimal dose determination for IND-enabling studies.

TSHA-106 for Angelman syndrome

We are developing TSHA-106 for the treatment of Angelman syndrome, a neurodevelopmental disorder caused by a maternal deficiency of the UBE3A gene. Angelman syndrome is characterized by profound developmental delay, ataxia and gait disturbance, sleep disorder, seizures, heightened anxiety, aggression and severe speech impairments. Angelman syndrome affects approximately one per 12,000 to 20,000 patients worldwide.

Angelman syndrome is an imprinting disorder in which the maternal gene is deficient and the paternal copy of UBE3A is intact but silenced by a long non-coding RNA, UBE3A antisense transcript, or UBE3A-ATS. Delivery of an ASO targeting UBE3A-ATS showed promising results in ameliorating Angelman syndrome symptoms in a transgenic mouse model.

We have in-licensed a novel gene replacement therapy from University of North Carolina. This novel construct is designed to express two isoforms of UBE3A mRNA from the same codon optimized transgene cassette and could potentially be a one-time treatment for the disease. The unique design feature allows short and long hUBE3A isoforms expression at a near-endogenous 3:1 (short/long) ratio, a feature that could help to support optimal therapeutic outcomes. Additionally, this construct uses human Synapsin 1 promoter, to limit UBE3A expression primarily in neurons, the primary therapeutic target for treating Angelman syndrome.

In a published study, this dual isoform expressing cassette was packaged into PHP.B capsids and administered by intracerebroventricular injections in neonatal mice models. This treatment significantly improved motor learning and innate behaviors in Angelman syndrome mice (PMID: 34676830). It rendered Angelman syndrome mice resilient to epileptogenesis and associated hippocampal neuropathologies induced by seizure kindling. These results demonstrated the feasibility, tolerability, and therapeutic potential for dual-isoform hUBE3A gene transfer in the treatment of AS in mice.

To advance these findings into translatable interventions, our collaborators packaged the dual isoform expressing cassette into AAV9 capsids and undertook animal proof of concept studies. Overall, these results are highly consistent with the published data describing neonatal ICV delivery of a similar dose of the PHP.B/hUBE3Aopt vector (PMID: 34676830) and support continued development. Ongoing and future work is focused on optimal dose and route of administration determination for IND enabling studies.

There are an estimated 55,000 patients with Angelman syndrome in the United States and Europe.

TSHA-114 for Fragile X Syndrome

We are developing TSHA-114 for the treatment of Fragile X syndrome, the most common single gene cause of autism and cognitive impairment, affecting about one in 6,000 individuals worldwide. Fragile X syndrome is diagnosed around three years of age and characterized by anxiety, aggression, hyperactivity, attention deficits and sleep and communication disruption.

Fragile X syndrome is caused by a pathological expansion of a CGG triplet repeat in the 5' untranslated region of the FMR1 gene. Expansion of the triplet above the normal 5–55 repeats to 200 or more causes hypermethylation of the gene promoter, and shutdown of transcription and translation of the encoded protein, fragile X mental retardation protein, or FMRP. The expanded repeat also induces formation of RNA: DNA heteroduplexes that induces epigenetic gene silencing. Although most patients with Fragile X syndrome do not express FMRP, some individuals with the full mutation produce low amounts of the protein (less than 10% of normal levels). FMRP expression in unaffected persons varies greatly from person to person. Current pharmacotherapeutic treatments for Fragile X syndrome are solely directed towards symptom relief.

We conducted proof of concept studies in animal models of Fragile X (Fmr1 KO) with TSHA-114. No significant adverse effects were observed in behavioral, serological or pathohistological markers up to 12 months after intrathecal administration of TSHA-114 in wild-type mice. TSHA-114 treated FMRKO showed widespread FMRP expression was observed throughout brain post administration. TSHA-114 treated FMRKO mice showed robust suppression of audiogenic seizures and normalization of fear conditioning behavior. In addition, assessment of circadian locomotor activity revealed restoration of hyperactivity and sleep. Assessment of transgene expression and behavioral responses in individual mice demonstrated correlations between the level of FMRP expression and potential drug efficacy.

The results from the study support continued development. Ongoing and future work is focused on optimal dose and route of administration determination for IND enabling studies.

There are an estimated 75,000 patients with Fragile X syndrome in the United States and Europe.

License Agreements

Research, Collaboration and License Agreement with The University of Texas Southwestern Medical Center

In November 2019, we entered into a research, collaboration and license agreement, or the UT Southwestern Agreement, with The Board of Regents of the University of Texas System on behalf of UT Southwestern, as amended in April 2020.

In connection with the UT Southwestern Agreement, we obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, we obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights. We are required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

On April 2, 2020, we amended the UT Southwestern Agreement to include the addition of another licensed product and certain indications, and a right of first refusal to us over certain patient dosing patents. No additional consideration was transferred in connection with this amendment. In March 2022, we and UT Southwestern mutually agreed to revise the payment schedules and current performance expectations of the current sponsored research agreements under the UT Southwestern Agreement and defer payments by fifteen months. In December 2023, we and UT Southwestern mutually agreed to terminate specific sponsored research agreements. There are no outstanding payments due for these terminated programs as of March 31, 2024.

In connection with the UT Southwestern Agreement, we issued to UT Southwestern 2,179,000 shares of our common stock. We do not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement, other than costs related to the maintenance of patents.

The UT Southwestern Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last valid claim of a licensed patent in such country for such licensed product. After the initial research term, we may terminate the agreement, on an indication-by-indication and licensed product-by-licensed product basis, at any time upon specified written notice to UT Southwestern. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. In December 2023, we transferred rights to specific indications back to UT Southwestern.

License Agreement with Abeona (CLN1 Disease)

In August 2020, we entered into a license agreement, or the Abeona CLN1 Agreement, with Abeona Therapeutics Inc., or Abeona. In connection with the Abeona CLN1 Agreement, we obtained an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses under certain patents, know-how and materials originally developed by the University of North Carolina at Chapel Hill and Abeona to research, develop, manufacture, have manufactured, use, and commercialize licensed products for gene therapy for the prevention, treatment, or diagnosis of CLN1 Disease (one of the forms of Batten disease) in humans.

Subject to certain obligations of Abeona, we are obligated to use commercially reasonable efforts to develop at least one product and commercialize at least one product in the United States.

In connection with the license grant, we paid Abeona a one-time upfront license fee of $3.0 million during fiscal year 2020. We are obligated to pay Abeona up to $26.0 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed product and high single-digit royalties on net sales of licensed products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the latest of the expiration or revocation or complete rejection of the last licensed patent covering such licensed product in the country where the licensed product is sold, the loss of market exclusivity in such country where the product is sold, or, if no licensed product exists in such country and no market exclusivity exists in such country, ten years from first commercial sale of such licensed product in such country. In addition, concurrent with the Abeona CLN1 Agreement, we entered into a purchase and reimbursement agreement with Abeona, pursuant to which we purchased specified inventory from Abeona and reimbursed Abeona for certain research and development costs previously incurred for total consideration of $4.0 million paid in fiscal year 2020.

In December 2021, our CTA filing for TSHA-118 for the treatment of CLN1 disease was approved by Health Canada and therefore triggered a regulatory milestone payment in connection with the Abeona CLN1 Agreement. We recorded $3.0 million within research and development expenses in the consolidated statements of operations for the year ended December 31, 2021. The milestone fee was paid in January 2022 and has been classified as an investing outflow in the condensed consolidated statements of cash flows for the three months ended March 31, 2022. No additional milestone payments were made or triggered during the three months ended March 31, 2024.

The Abeona CLN1 Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product in such country. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. We may terminate the agreement for convenience upon specified prior written notice to Abeona.

License Agreement with Abeona (Rett Syndrome)

In October 2020, we entered into a license agreement, or the Abeona Rett Agreement, with Abeona pursuant to which we obtained an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses under certain patents, know-how and materials originally developed by the University of North Carolina at Chapel Hill, the University of Edinburgh and Abeona to research, develop, manufacture, have manufactured, use, and commercialize licensed products for gene therapy and the use of related transgenes for Rett syndrome.

Subject to certain obligations of Abeona, we are required to use commercially reasonable efforts to develop at least one licensed product and commercialize at least one licensed product in the United States.

In connection with the Abeona Rett Agreement, we paid Abeona a one-time upfront license fee of $3.0 million during fiscal year 2020. We are obligated to pay Abeona up to $26.5 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed product and high single-digit royalties on net sales of licensed products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the latest of the expiration or revocation or complete rejection of the last licensed patent covering such licensed product in the country where the licensed product is sold, the loss of market exclusivity in such country where the product is sold, or, if no licensed product exists in such country and no market exclusivity exists in such country, ten years from first commercial sale of such licensed product in such country.

In March 2022, our CTA filing for TSHA-102 for the treatment of Rett Syndrome was approved by Health Canada and therefore triggered a regulatory milestone payment of $1.0 million in connection with the Rett Agreement, which was paid in July 2022. In May 2023, we dosed the first patient with TSHA-102 in the Phase 1/2 REVEAL trial evaluating the safety and preliminary efficacy of TSHA-102 in adult patients with Rett syndrome and therefore triggered a milestone payment of $3.5 million in connection with the Rett Agreement, which was paid in August 2023. No additional milestone payments were made during the three months ended March 31, 2024.

The Abeona Rett Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product in such country. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. We may terminate the agreement for convenience upon specified prior written notice to Abeona.

Option Agreement with Astellas

On October 21, 2022, or the Effective Date, we entered into an Option Agreement, or the Option Agreement, with Audentes Therapeutics, Inc. (d/b/a Astellas Gene Therapy), or Astellas.

TSHA-120 Giant Axonal Neuropathy

Under the Option Agreement, we granted to Astellas an exclusive option to obtain an exclusive, worldwide, royalty and milestone-bearing right and license (A) to research, develop, make, have made, use, sell, offer for sale, have sold, import, export and otherwise exploit, or, collectively, Exploit or the Exploitation, the product known, as of the Effective Date, as TSHA-120, or the 120 GAN Product, and any backup products with respect thereto for use in the treatment of GAN or any other gene therapy product for use in the treatment of GAN that is controlled by us or any of our affiliates or with respect to which we or any of our affiliates controls intellectual property rights covering the Exploitation thereof, or a GAN Product, and (B) under any intellectual property rights controlled by us or any of our affiliates with respect to such Exploitation, or the GAN Option. Subject to certain extensions, the GAN Option was exercisable from the Effective Date through a specified period of time following Astellas' receipt of (i) the formal minutes from the Type B end-of-Phase 2 meeting between us and the FDA in response to our meeting request sent to the FDA on September 19, 2022 for the 120 GAN Product, (ii) all written feedback from the FDA with respect to the Type B end-of-Phase 2 Meeting, and (iii) all briefing documents sent by us to the FDA with respect to the Type B end-of-Phase 2 Meeting. Following the receipt of Type C meeting feedback from the FDA regarding a registrational path for TSHA-120 in September 2023, Astellas elected not to exercise the GAN Option, and we recognized revenue related to this expiration during the third quarter of 2023.

TSHA-102 Rett Syndrome

Under the Option Agreement, we also granted to Astellas an exclusive option to obtain an exclusive, worldwide, royalty and milestone-bearing right and license (A) to Exploit any Rett Product (as defined below), and (B) under any intellectual property rights controlled by us or any of our affiliates with respect to such Exploitation, or the Rett Option. Subject to certain extensions, the Rett Option is exercisable from the Effective Date through a specified period of time following Astellas' receipt of (1) certain clinical data from the female pediatric trial and (2) certain specified data with respect to TSHA-102, or the Rett Option Period related to (i) the product known, as of the Effective Date, as TSHA-102 and any backup products with respect thereto for use in the treatment of Rett syndrome, and (ii) any other gene therapy product for use in the treatment of Rett syndrome that is controlled by us or any of our affiliates or with respect to which we or any of our affiliates controls intellectual property rights covering the Exploitation thereof, or a Rett Product.

The parties have agreed that, if Astellas exercises an Option, the parties will, for a specified period, negotiate a license agreement in good faith on the terms and conditions outlined in the Option Agreement, including payments by Astellas of a to-be-determined upfront payment, certain to-be-determined milestone payments, and certain to-be-determined royalties on net sales of GAN Products and/or Rett Products, as applicable.

Components of Results of Operations

Revenue

Revenue for the three months ended March 31, 2024 was derived from the Astellas Transactions. We recognize revenue as research and development activities related to our Rett program are performed. Revenue related to the material rights associated with the Rett Option and the GAN Option must be recognized at a point in time when the options are exercised or the option period expires. In September 2023, Astellas elected not to exercise the GAN Option, therefore we recognized revenue related to the GAN Option during the year ended December 31, 2023.

To date, we have not recognized any revenue from product sales, and we do not expect to generate any revenue from the sale of products, if approved, in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, or license agreements with third parties, we may generate revenue in the future from product sales. However, there can be no assurance as to when we will generate such revenue, if at all.

Operating Expenses

Research and Development Expenses

Research and development expenses primarily consist of clinical and preclinical development of our product candidates and discovery efforts, including conducting preclinical studies, manufacturing development efforts, preparing for and conducting clinical trials and activities related to regulatory filings for our product candidates. Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods or services are received. Costs incurred in obtaining technology licenses through asset acquisitions are charged to research and

development expense if the licensed technology has not reached technological feasibility and has no alternative future use. Research and development expenses include or could include:

- employee-related expenses, including salaries, bonuses, benefits, stock-based compensation, severance costs and other related costs for those employees involved in research and development efforts;
- license maintenance fees and milestone fees incurred in connection with various license agreements;
- external research and development expenses incurred under agreements with consultants, contract research organizations, or CROs, investigative sites and consultants to conduct our preclinical studies;
- costs related to manufacturing material for our preclinical studies and clinical trials, including fees paid to contract manufacturing organizations, or CMOs;
- laboratory supplies and research materials;
- costs related to compliance with regulatory requirements; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, maintenance of facilities, insurance and equipment.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We reduced our research and development and general and administrative spend from 2022 to 2023 but have increased and expect to continue to increase our research and development expenses with respect to the Rett clinical trials for the foreseeable future as we continue the development of TSHA-102 and manufacturing processes and conduct discovery and research activities for our preclinical programs. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments as to each product candidate's commercial potential. We will need to raise substantial additional capital in the future. Our clinical development costs are expected to increase significantly as we commence clinical trials. Our future expenses may vary significantly each period based on factors such as:

- expenses incurred to conduct preclinical studies required to advance our product candidates into clinical development;
- per patient trial costs, including based on the number of doses that patients received;
- the number of patients who enroll in each trial;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the phase of development of the product candidate;
- third-party contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- the ability to manufacture our product candidates;
- regulators or institutional review boards requiring that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks; and

- the efficacy and safety profile of our product candidates.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for personnel in executive and administrative functions, including stock-based compensation, severance costs, travel expenses and recruiting expenses. Other general and administrative expenses include professional fees for legal, consulting, accounting and audit and tax-related services and insurance costs.

We anticipate that certain of our general and administrative expenses will decrease in the future as a result of the reductions in our headcount in 2022 and 2023 to support our infrastructure and focus on our Rett program. We also anticipate that our general and administrative expenses may increase in the future as a result of payments for accounting, audit, legal, consulting services, costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer liability insurance, investor and public relations activities and other expenses associated with operating as a public company to support planned future Rett program development.

Other Income (Expense)

Other income (expense) consists primarily of dividends earned from our money market fund and interest income on our cash and cash equivalents, interest expense on borrowings under the Trinity Term Loan, and non-cash changes in the fair value of our outstanding warrant liability and the Trinity Term Loan.

Results of Operations

Results of Operations for the Three Months ended March 31, 2024 and 2023

The following table summarizes our results of operations for the three months ended March 31, 2024 and 2023 (in thousands):

	For the Three Months Ended March 31,	
	2024	2023
Revenue	$ 3,411	$ 4,706
Operating expenses:		
Research and development	20,657	12,514
General and administrative	7,084	8,751
Total operating expenses	27,741	21,265
Loss from operations	(24,330)	(16,559)
Other income (expense):		
Change in fair value of warrant liability	(337)	—
Change in fair value of term loan	(1,053)	—
Interest income	1,693	319
Interest expense	(29)	(1,374)
Other expense	(5)	(8)
Total other income (expense), net	269	(1,063)
Net loss	$ (24,061)	$ (17,622)

Revenue

Revenue related to the Astellas Transactions was $3.4 million for the three months ended March 31, 2024, compared to $4.7 million for the three months ended March 31, 2023. The revenue recorded is the result of Rett research and development activities performed during the respective first quarter of 2024 and 2023.

Research and Development Expenses

Research and development expenses were $20.7 million for the three months ended March 31, 2024, compared to $12.5 million for the three months ended March 31, 2023. The $8.2 million increase was primarily driven by a $7.6 million increase in GMP batch activities during the three months ended March 31, 2024, which is representative of the intended commercial manufacturing process for TSHA-102 in Rett syndrome. Additionally, clinical trial expenses increased due to ongoing activities in the REVEAL adolescent/adult and pediatric trials.

General and Administrative Expenses

General and administrative expenses were $7.1 million for the three months ended March 31, 2024, compared to $8.8 million for the three months ended March 31, 2023. The decrease of $1.7 million was due to reduced general and administrative compensation as a result of lower headcount and reduced consulting and professional fees.

Other Income (Expense)

Change in fair value of warrant liability

Change in fair value of warrant liability was a non-cash expense totaling $0.3 million for the three months ended March 31, 2024 related to the SSI Warrants (as defined below).

Change in fair value of term loan

We elected the fair value option for the Trinity Term Loan and changes to fair value, other than changes that are directly attributable to instrument-specific credit risk, are recorded as a component of other income (expense). The change in fair value was $1.1 million for the three months ended March 31, 2024.

Interest Income

Interest income was $1.7 million for the three months ended March 31, 2024 compared to $0.3 million for the three months ended March 31, 2023. The increase in income is primarily attributable to dividends earned from our money market fund and interest earned on our savings account following the investment of proceeds raised in our August 2023 Private Placement (as defined below).

Interest Expense

Interest expense was less than $0.1 million for the three months ended March 31, 2024, compared to $1.4 million for the three months ended March 31, 2023. The decrease of approximately $1.3 million was primarily attributable to interest expense incurred under the Term Loan Agreement for the three months ended March 31, 2023.

Liquidity and Capital Resources

Overview

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses. As of March 31, 2024, we had cash and cash equivalents of $124.0 million. We have funded our operations primarily through equity financings, raising an aggregate of $589.0 million in gross proceeds from equity financings, including from pre-IPO private placements of convertible preferred stock, our IPO, and subsequent sales of common stock in public and private securities offerings, our term loans and the Astellas Transactions.

On the Trinity Closing Date, we entered into the Trinity Term Loan Agreement, by and among us, the Trinity Lenders, and Trinity. The Trinity Term Loan Agreement provided for, on the Trinity Closing Date, $40.0 million aggregate principal amount of term loans, or, collectively, the Trinity Term Loans. We drew the Trinity Term Loans in full on the Trinity Closing Date. The interest rate applicable to the Trinity Term Loans is the greater of (a) the Wall Street Journal Prime Rate plus 4.50% or (b) 12.75% per annum. The Trinity Term Loans are interest only from the Trinity Closing Date through 36 months from the Trinity Closing Date, which may be extended to 48 months from the Trinity Closing Date upon the satisfaction of certain milestones set forth in the Trinity Term Loan Agreement, after which we are required to pay equal monthly installments of principal through November 13, 2028, or the Maturity Date. The Trinity Term Loans may be prepaid in full (i) from the Trinity Closing Date through November 13, 2024, with payment of a 3.00% prepayment premium, (ii) from November 13, 2024 through November 13, 2025, with payment of a 2% prepayment premium, and (iii) from November 13, 2025 through, but excluding, the Maturity Date, with payment of a 1% prepayment premium. On the Trinity Closing Date, we paid Trinity a commitment fee of 1.00% of the original principal amount of the Trinity Term Loans. Upon repayment in full of the Trinity Term Loans, we will pay Trinity an end of term payment equal to 5.00% of the original principal amount of the Trinity Term Loans.

The obligations under the Trinity Term Loan Agreement are secured by a perfected security interest in all of our assets except for certain customarily excluded property pursuant to the terms of the Trinity Term Loan Agreement. There are no financial covenants and no warrants associated with the Trinity Term Loan Agreement. The Trinity Term Loan Agreement contains various covenants that limit our ability to engage in specified types of transactions without the consent of Trinity and the Trinity Lenders which include, among others, incurring or assuming certain debt; merging, consolidating or acquiring all or substantially all of the capital stock or property of another entity; changing the nature of our business; changing our organizational structure or type; licensing, transferring or

disposing of certain assets; granting certain types of liens on our assets; making certain investments; and paying cash dividends. The Trinity Term Loan Agreement also contains customary representations and warranties, and also includes customary events of default, including payment default, breach of covenants, change of control, and material adverse effects. Upon the occurrence of an event of default, a default interest rate of an additional 5% per annum may be applied to the outstanding loan balances, and the Trinity Lenders may declare all outstanding obligations immediately due and payable and exercise all of its rights and remedies as set forth in the Trinity Term Loan Agreement and under applicable law. The proceeds of the Trinity Term Loans were used to repay our obligations under the Term Loan Agreement with Silicon Valley Bank in full. The Term Loan Agreement with Silicon Valley Bank was terminated concurrently with entry into the Trinity Term Loan Agreement.

On October 5, 2021, we filed a shelf registration statement on Form S-3 with the SEC in relation to the registration of common stock, preferred stock, debt securities, warrants and units or any combination thereof up to a total aggregate offering price of $350.0 million. We also simultaneously entered into a Sales Agreement, or the Sales Agreement with SVB Leerink LLC and Wells Fargo Securities, LLC, or the Sales Agents, pursuant to which we may issue and sell, from time to time at our discretion, shares of our common stock having an aggregate offering price of up to $150.0 million through the Sales Agents. In March 2022, we amended the Sales Agreement to, among other things, include Goldman Sachs & Co. LLC as an additional Sales Agent. In April 2022, we sold 2,000,000 shares of common stock pursuant to the Sales Agreement and received net proceeds of $11.6 million. No other shares of common stock have been issued and sold pursuant to the Sales Agreement as of March 31, 2024.

On October 21, 2022, we entered into the Option Agreement with Astellas granting Astellas an exclusive option to obtain exclusive, worldwide, royalty and milestone-bearing rights and licenses related to TSHA-120 and TSHA-102. As partial consideration for the rights granted to Astellas under the Option Agreement, Astellas paid us a one-time payment in the amount of $20.0 million, or the Upfront Payment, in November 2022.

Also on October 21, 2022, we entered into a securities Purchase Agreement with Astellas, or the Astellas Securities Purchase Agreement, and together with the Option Agreement, the Astellas Transactions, pursuant to which we agreed to issue and sell to Astellas in a private placement, or the Astellas Private Placement, an aggregate of 7,266,342 shares of our common stock, or the Astellas Private Placement Shares, for aggregate proceeds of approximately $30.0 million. The Astellas Private Placement closed on October 24, 2022. Pursuant to the Astellas Securities Purchase Agreement, in connection with the Astellas Private Placement, Astellas has the right to designate one individual to attend all meetings of the Board in a non-voting observer capacity. We also granted Astellas certain registration rights with respect to the Astellas Private Placement Shares.

On October 26, 2022, we entered into an underwriting agreement, or the Underwriting Agreement, with Goldman Sachs & Co. LLC, or the Underwriter, to issue and sell 14,000,000 shares of our common stock, par value $0.00001 per share, in an underwritten public offering, or the Follow-on Offering, pursuant to an effective registration statement on Form S-3 and a related prospectus and prospectus supplement. The offering price to the public was $2.00 per share and the Underwriter purchased the shares from us pursuant to the Underwriting Agreement at a price of $1.88 per share. In addition, we granted the Underwriter an option to purchase, for a period of 30 days, up to an additional 2,100,000 shares of our common stock. The Follow-on Offering closed on October 31, 2022 and we received net proceeds of $26.0 million after deducting underwriting discounts, commissions and offering expenses. On November 10, 2022, the Underwriter exercised their option to purchase an additional 765,226 shares of our common stock and we received net proceeds of $1.4 million after deducting underwriting discounts and commissions.

In April 2023, we entered into a securities purchase agreement, or the SSI Securities Purchase Agreement, with two affiliates of SSI Strategy Holdings LLC, or SSI, named therein, or the SSI Investors, pursuant to which we agreed to issue and sell to the SSI Investors in a private placement, or the SSI Private Placement, 705,218 shares of our common stock, or the SSI Shares, and warrants, or the SSI Warrants, to purchase an aggregate of 525,000 shares of our common stock, or the Warrant Shares. SSI provides certain consulting services to us. Each SSI Warrant has an exercise price of $0.7090 per Warrant Share, which was the closing price of our common stock on the Nasdaq Global Market on April 4, 2023. The SSI Warrants issued in the SSI Private Placement provide that the holder of the SSI Warrants will not have the right to exercise any portion of its SSI Warrants until the achievement of certain clinical and regulatory milestones related to our clinical programs. The SSI Private Placement closed on April 5, 2023. Gross proceeds of the SSI Private Placement were $0.5 million.

On August 14, 2023, we entered into a securities purchase agreement, or the August 2023 Securities Purchase Agreement, with certain institutional and other accredited investors, or the Purchasers, pursuant to which we agreed to sell and issue to the Purchasers in a private placement transaction, or the August 2023 Private Placement, that closed on August 16, 2023: (i) 122,412,376 shares of our common stock and (ii) with respect to certain Purchasers, pre-funded warrants, or the Pre-Funded Warrants, to purchase 44,250,978 shares of common stock in lieu of shares of common stock. The closing of the August 2023 Private Placement, or the Closing, occurred on August 16, 2023. The total gross proceeds to us at the Closing were $150.0 million, and after deducting placement agent commissions and offering expenses payable by us, net proceeds were $140.3 million.

Funding Requirements

To date, we have not generated any revenues from the commercial sale of approved drug products, and we do not expect to generate substantial revenue for at least the next few years. If we fail to complete the development of our product candidates in a timely manner or fail to obtain their regulatory approval, our ability to generate future revenue will be compromised. We do not know when, or if, we will generate any revenue from our product candidates, and we do not expect to generate significant revenue unless and until we obtain regulatory approval of, and commercialize, our product candidates. We reduced spending in 2023, and anticipate such reductions will continue in 2024, as a result of our decision to discontinue development of our GAN clinical program. We have increased and expect to continue to increase our research and development expenses, particularly with respect to the Rett clinical trials, for the foreseeable future as we continue the development of our product candidates and manufacturing processes and conduct discovery and research activities for our preclinical programs. If we obtain approval for any of our product candidates, we expect to incur significant commercialization expenses related to sales, marketing, manufacturing and distribution. We anticipate that we will need substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

As of March 31, 2024, our material cash requirements consisted of $31.0 million in total lease payments under our noncancelable leases for equipment, laboratory space and office space. These leases are described in further detail in Note 5 to our unaudited condensed consolidated financial statements located in "Part I – Financial Information, Item 1. Financial Statements" in this Quarterly Report on Form 10-Q. Our most significant purchase commitments consist of approximately $9.6 million in cancellable purchase obligations to our CROs and other clinical trial vendors.

We believe that our existing cash and cash equivalents will enable us to fund our operating expenses and capital requirements into 2026. We will require additional capital to fund the research and development of our product candidates, to fund our manufacturing activities, to fund precommercial activities of our programs and for working capital and general corporate purposes. The assessment of our ability to meet our future obligations is inherently judgmental, subjective and susceptible to change.

Because of the numerous risks and uncertainties associated with research, development and commercialization of biological products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for TSHA-102 and any current and future product candidates that we advance;
- our ability to access sufficient additional capital on a timely basis and on favorable terms;
- the extent to which we develop, in-license or acquire other product candidates and technologies in our gene therapy product candidate pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development;
- the number and development requirements of product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales, and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the costs incurred in defending ourselves in any legal proceedings that we may be subject to;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain

marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available in the near term, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the terms of these equity securities or this debt may restrict our ability to operate. The Trinity Term Loan Agreement contains negative covenants, including, among other things, restrictions on indebtedness, liens investments, mergers, dispositions, prepayment of other indebtedness and dividends and other distributions. Any future additional debt financing and equity financing, if available, may involve agreements that include covenants limiting and restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, entering into profit-sharing or other arrangements or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

Cash Flows

The following table shows a summary of our cash flows for the three months ended March 31, 2024 and 2023 (in thousands):

	For the Three Months Ended March 31,	
	2024	2023
Net cash used in operating activities	$ (19,798)	$ (20,185)
Net cash used in investing activities	(140)	(3,900)
Net cash used in financing activities	(22)	(370)
Net change in cash, cash equivalents and restricted cash	$ (19,960)	$ (24,455)

Operating Activities

For the three months ended March 31, 2024, our net cash used in operating activities of $19.8 million primarily consisted of a net loss of $24.1 million, primarily attributable to our spending on research and development expenses. The net loss of $24.1 million was partially offset by adjustments for non-cash items, primarily stock-based compensation expense of $3.2 million and other non-cash items of $1.0 million, net. Additional cash provided by operating assets and liabilities of $0.3 million was primarily attributable to an increase in accounts payable of $4.0 million and offset by a decrease in deferred revenue of $3.4 million.

For the three months ended March 31, 2023, our net cash used in operating activities of $20.2 million primarily consisted of a net loss of $17.6 million, primarily attributable to our spending on research and development expenses. The net loss of $17.6 million was partially offset by adjustments for non-cash items, primarily stock-based compensation expense of $1.7 million. Additional cash used in operating activities of $5.0 million was primarily due to a decrease in deferred revenue.

Investing Activities

During the three months ended March 31, 2024, investing activities used $0.1 million of cash primarily attributable to the purchase of lab equipment. During the three months ended March 31, 2023, investing activities used $3.9 million of cash primarily attributable to capital expenditures related to the close-out of our in-house manufacturing facility project.

Financing Activities

During the three months ended March 31, 2024, financing activities used less than $0.1 million of cash, which is primarily attributable to the payment of lease financing obligations which were partially offset by ESPP contributions. During the three months ended March 31, 2023, financing activities used $0.4 million of cash, which is primarily attributable to the payment of shelf registration costs and other financing transactions.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical Accounting Policies and Significant Judgments and Estimates

There were no material changes to our critical accounting policies that are disclosed in our audited consolidated financial statements for the year ended December 31, 2023 filed with the SEC on March 19, 2024.

Recent Accounting Pronouncements

See Note 2 to our unaudited condensed consolidated financial statements located in "Part I – Financial Information, Item 1. Financial Statements" in this Quarterly Report on Form 10-Q for a description of recent accounting pronouncements applicable to our condensed consolidated financial statements.

Emerging Growth Company and Smaller Reporting Company Status

In April 2012, the Jumpstart Our Business Startups Act of 2012, or JOBS Act, was enacted. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We elected the extended transition period for complying with new or revised accounting standards, which delays the adoption of these accounting standards until they would apply to private companies.

In addition, as an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on financial statements.

We may take advantage of these provisions until we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) December 31, 2025, (ii) the last day of the fiscal year in which we have more than $1.235 billion in total annual gross revenues, (iii) the date on which we are deemed to be a "large accelerated filer" under the rules of the SEC, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, or (iv) the date on which we have issued more than $1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. We have taken advantage of certain reduced reporting requirements in this Quarterly Report on Form 10-Q and our other filings with the SEC. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

We are also a "smaller reporting company," meaning that the market value of our shares held by non-affiliates is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act and are not required to provide the information required under this Item.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), as of

the end of the period covered by this Form 10-Q. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that as of March 31, 2024, our disclosure controls and procedures were effective to provide reasonable assurance that the information required to be disclosed by us in this Form 10-Q was (a) reported within the time periods specified by SEC rules and regulations, and (b) communicated to our management, including our Chief Executive Officer and Chief Financial Officer, to allow timely decisions regarding any required disclosure.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting identified in management's evaluation pursuant to Rules 13a-15(d) or 15d-15(d) of the Exchange Act during the period covered by this Quarterly Report on Form 10-Q that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on Effectiveness of Internal Controls

In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable, not absolute, assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs. Our management, including our Chief Executive Officer and Chief Financial Officer, believes that our disclosure controls and procedures and internal control over financial reporting are designed to provide reasonable assurance of achieving their objectives and are effective at the reasonable assurance level. However, our management does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all errors and all fraud.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not subject to any material legal proceedings. From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors.

Our business is subject to risks and events that, if they occur, could adversely affect our financial condition and results of operations and the trading price of our securities. In addition to the other information set forth in this quarterly report on Form 10-Q, you should carefully consider the factors described in Part I, Item 1A. "Risk Factors" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the Securities and Exchange Commission on March 19, 2024.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

(a) Recent Sales of Unregistered Equity Securities

None.

(b) Use of Proceeds

None.

(c) Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index are either filed or furnished with this report or incorporated herein by reference.

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-39536), filed with the Securities and Exchange Commission on September 29, 2020).
3.2	Amended and Restated Bylaws (incorporated by reference to Exhibit 3.4 to the Company's Current Report on Form 8-K (File No. 001-39536), filed with the Securities and Exchange Commission on September 29, 2020).
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-39536), filed with the Securities and Exchange Commission on November 15, 2023).
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1#	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2#	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101).

* Filed herewith.

These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Taysha Gene Therapies, Inc.

Date: May 14, 2024

By: /s/ Sean Nolan

Sean Nolan
Chief Executive Officer
(Principal Executive Officer)

Date: May 14, 2024

By: /s/ Kamran Alam

Kamran Alam
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Sean Nolan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Taysha Gene Therapies, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in exchange act rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 14, 2024

By: /s/ Sean Nolan

Sean Nolan
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kamran Alam, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Taysha Gene Therapies, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in exchange act rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 14, 2024

By: /s/ Kamran Alam

Kamran Alam
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Sean Nolan, Chief Executive Officer of Taysha Gene Therapies, Inc. (the "Company") hereby certifies that, to the best of his knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the period ended March 31, 2024, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

(2) The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 14, 2024

By: /s/ Sean Nolan

Sean Nolan
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Kamran Alam, Chief Financial Officer of Taysha Gene Therapies, Inc. (the "Company") hereby certifies that, to the best of his knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the period ended March 31, 2024, to which this Certification is attached as Exhibit 32.2 (the "Periodic Report") fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

(2) The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 14, 2024

By: /s/ Kamran Alam

Kamran Alam
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)