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Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch

Aug. 17, 2023 6:42 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | ACAD, AVXL | 7 Comments | 3 Likes



Terry Chrisomalis

Investing Group Leader

Summary

- Taysha Gene Therapies has made significant progress with its gene therapy, TSHA-102, for the treatment of Rett Syndrome.
- Positive results were observed in the first patient treated with TSHA-102, showing improvement in key efficacy measures.
- The company is also advancing another gene therapy candidate, TSHA-120, for the treatment of Giant Axonal Neuropathy.
- It is expected that the Rett Syndrome market will hit \$515.06 million by 2029.
- I am Terry Chrisomalis, a long term Biotech investor with a degree in Applied Science. I am the leader of the investing group [Biotech Analysis Central](#), where I analyze high risk/high reward ideas.



nuttapong punna/iStock via Getty Images

Taysha Gene Therapies (NASDAQ:[TSJA](#)) has made great progress recently in advancing its gene therapy known as TSHA-102 for the treatment of patients with Rett Syndrome. Despite only having data from one patient so far, with a second expected to start dosing in Q3 of 2023, the data shown thus far is impressive. The biotech noted that it intends to provide quarterly updates for its clinical programs, thus data from the second patient recruited into this phase 1/2 REVEAL trial, could be released by Q4 of 2023. If the second patient achieves a similar or better outcome than the first patient, then it will be confirmation that TSHA-102 may be highly suitable to treat patients with Rett Syndrome.

Besides such a catalyst in the coming months, this biotech has another gene therapy candidate being advanced, which is TSHA-120. This other gene therapy candidate is being advanced for the treatment of patients with Giant Axonal Neuropathy [GAN].

Taysha is [gearing up to meet with the FDA in Q3 of 2023](#), whereby it and the agency will discuss a potential regulatory pathway forward for the use of TSHA-120 for the treatment of patients with GAN. Should this meeting conclude in a successful manner, then the company may be given the green light to initiate a pivotal study for this program. This remains to be seen, but this is another catalyst for traders/investors to look forward to. Lastly, it was able to obtain a \$150 million private placement financing to boost its cash on hand. It believes that with this recent financing transaction, that it would have enough cash on hand to fund its operations into Q3 of 2025.

TSHA-102 Achieves Huge Improvement In First Patient With Rett Syndrome

Taysha Gene Therapies is advancing the use of gene therapy candidate TSHA-102 for the treatment of patients with Rett Syndrome. **Rett Syndrome** is a rare genetic neurological disorder by which severe impairment occurs in every aspect of an affected person's life. It affects many functions of life such as: Speech, coordination, and motor movement. It occurs as a result of a gene mutation, which is first recognized in infancy. While this disorder primarily affects girls, it is also being identified in many males as well. Other problems associated with this disease are muscle movements and communication skills. The thing is that most babies with Rett Syndrome develop as expected in the first 6 months of their life, however, they then lose such skills previously learned. They lose the ability to be able to communicate properly, crawl, walk or use their hands as needed. It is expected that the **Rett Syndrome market will hit \$515.06 million by 2029**. This is a pretty good market opportunity for any biotech that can capture this market. Thus far, the whole market lies at the foot of **Acadia Pharmaceuticals (ACAD)**, which **received FDA approval of its drug Daybue** for the treatment of Rett Syndrome in adult and pediatric patients 2 years of age and older. One thing to note is that Daybue is the first and only drug approved for the treatment of this patient population. However, another biotech with potential to capture this Rett Syndrome indication might be **Anavex Life Sciences (AVXL)**. That's because it is gearing up to report top-line data from its potentially pivotal **Anavex2-73-RS-003 phase 2/3 EXCELLENCE pediatric clinical trial**. Top-line results from this study are expected to be released in the 2nd half of 2023. If this study succeeds, then this might be another possible competitor that Taysha Gene Therapies may have to ultimately contend with.

The thing is that the positive results that Taysha Gene Therapies was able to release, came from the **phase 1/2 REVEAL trial**, which used gene therapy TSHA-102 for the treatment of 1 patient with Rett Syndrome. Despite this only being one patient, it was noted that they had achieved improvement in key efficacy measures. Such clinical efficacy measures where improvement was achieved is as follows:

- **Rett Syndrome Behavior Questionnaire [4-weeks post treatment]**
- **Clinical Global Impression - Severity [CGI-S]**
- **Clinical Global Impression - Improvement [CGI-I]**

It was also noted that TSHA-102 was well tolerated, with no treatment emergent serious adverse events at 6-weeks. All these efficacy measures are great, but what is really outstanding is what this patient was able to accomplish after being given this therapy. Before this therapy their motor function was gone in early childhood, needed help to sit in an upright position and other issues. Once this patient was given TSHA-102, they were able to achieve a multitude of functions, such as the following:

- ***Improved breathing patterns***
- ***Motor skills***
- ***Vocalization***
- ***Being able to sit without help for first time in over a decade***
- ***Holding an object in a steady manner***

While this was only observed in one patient thus far, it may be possible to see such marked improvement in another patient.

One thing to note is that the advancement of TSHA-102 for the phase 1/2 REVEAL study is only being done in adult patients. That is, the company is already preparing to expand the use of this gene therapy for the pediatric patient population. It has already made a move to move into this specific pediatric patient population through several advancements in the recent year. It has already **received clearance from the FDA** to initiate TSHA-102 in pediatric patients with Rett Syndrome in the United States. Not only that, but it has also already submitted a Clinical Trial Authorization [CTA] to the MHRA for TSHA-102 for pediatric patients with Rett Syndrome. Thus, the potential here lies with the ability to eventually expand to the entire Rett Syndrome patient population and not just in adults.

Financials

According to the **10-Q SEC Filing**, Taysha Gene Therapies had cash and cash equivalents of \$45.1 million as of June 30, 2023. It was able to receive gross **proceeds of \$150 million from a Private Placement Financing [PIPE]** from new and existing investors. With this newly obtained financing, plus the cash on hand it already had, it believes that it will have enough to fund its operations into Q3 of 2025. It should be good on cash for now, but I wouldn't preclude the possibility that it might have to raise additional cash later on. That's because it **intends to release quarterly updates on clinical data**. With the second patient dosed and data coming within the coming months, it's possible that this might cause the stock price to trade higher. This or any other positive catalyst might make management consider raising additional cash. Such a raise is not guaranteed to happen, but quite possible nonetheless.

Risks To Business

There are several risks that investors should be aware of before investing in Taysha Gene Therapies. The first risk to consider would be with respect to the ongoing phase 1/2 REVEAL study, which is using TSHA-102 for the treatment of adults with Rett Syndrome. That's because additional results from the second patient in the study are expected to be released at least by Q4 of 2023. Despite the first patient seeing improvement with multiple efficacy measures with TSHA-102 for Rett Syndrome, there is no guarantee that the second patient will achieve a similar or superior outcome. A second risk to consider would then be about the possible expansion opportunity with respect to the Rett Syndrome patient population.

The company has already taken steps to initiate a few early-stage studies using TSHA-102 in pediatric patients with this disorder. There is no assurance that clinical data generated in adults will then translate over to the pediatric patient population. A third and final risk would be with respect the upcoming FDA meeting in Q3 of 2023, which is slated to discuss the potential regulatory approval pathway of using TSHA-120 for the treatment of patients with GAN. Taysha believes that it has the potential to a regulatory pathway in advancing this candidate for this patient population. However, it won't truly know what the FDA wants until it meets with the agency during this quarter. A risk here would be that the FDA may require additional patients or a new study, before even considering allowing a pivotal one.

Conclusion

Taysha Gene Therapies has done well to advance the use of TSHA-102 for the treatment of adults with Rett Syndrome. However, further confirmation is truly needed before knowing whether or not there is a good chance for the company to eventually receive FDA approval for it. At least, the biotech has already thought about expanding the use of TSHA-102 for the pediatric patient populations, which will help it target a larger chunk of the Rett Syndrome market. This was **done through the clearance of the Investigational New Drug [IND] application of TSHA-102** and submission of the Clinical Trial Application [CTA] to the United Kingdom [UK] Medicines and Healthcare products Regulatory Agency [MHRA] for TSHA-102 in pediatric patients with Rett Syndrome.

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This article was written by



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He is the author of the investing group [Biotech Analysis Central](#) which contains a library of 600+ Biotech investing articles, a model portfolio of 10+ small and mid-cap stocks with deep analysis for each, live chat, and a range of analysis and news reports to help Healthcare investors make informed decisions. [Learn more](#).

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Taysha Gene Therapies: TSHA-102's Potential Is A 'Buy'

Nov. 03, 2023 7:41 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | 4 Likes

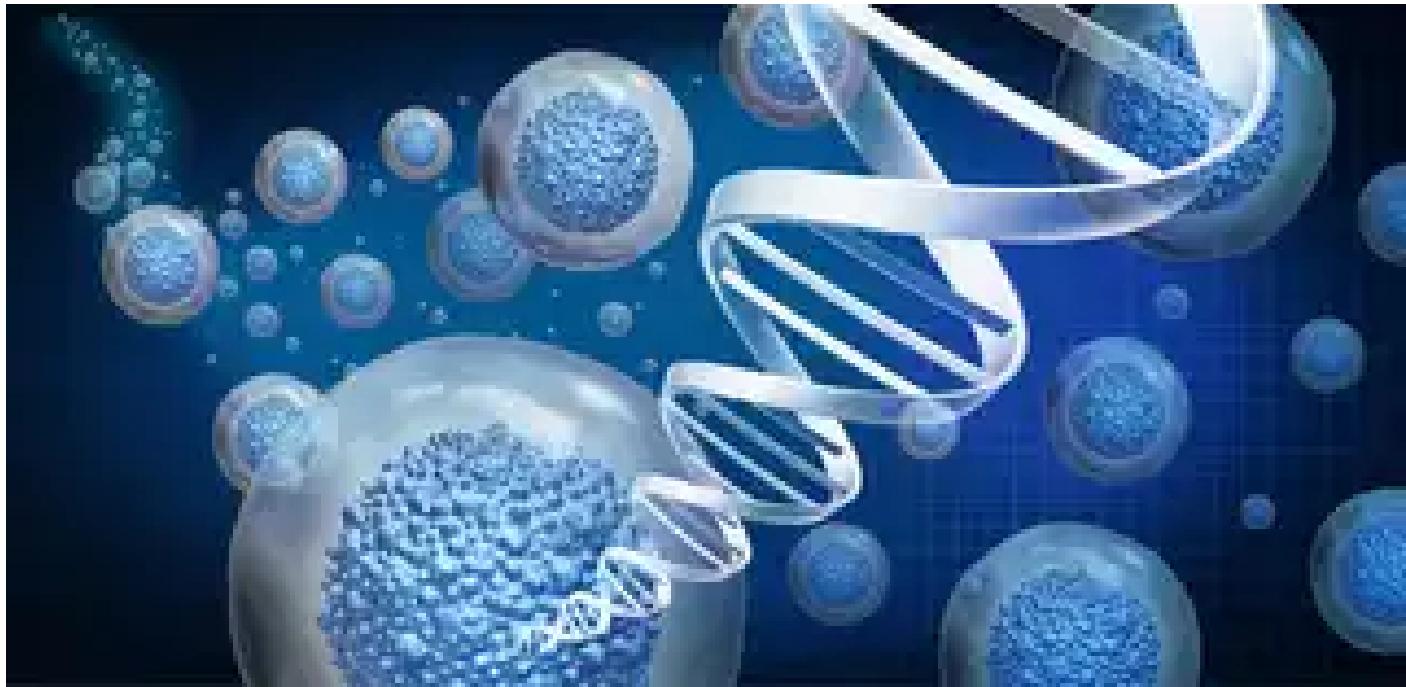


Myriam Alvarez

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Summary

- Taysha Gene Therapies is a biotech stock focused on developing gene therapies for monogenic CNS diseases, with its lead candidate TSHA-102 targeting Rett Syndrome.
- The gene therapy market is projected to grow from \$15.46 billion in 2022 to \$82.24 billion by 2032, highlighting a shift towards personalized medicine.
- TSHA-102 has received the FDA's Fast Track Designation, expediting its Phase 1/2 trial with a roadmap to treat the first pediatric patient by Q1 2024.
- Despite the potential, TSHA has significant financial constraints and regulatory hurdles to overcome, emphasizing its speculative nature.
- I conclude that TSHA might be a good speculative buy, considering the revenue potential of TSHA-102 if it is successfully developed and commercialized.



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Taysha Gene Therapies, Inc. (NASDAQ:[TSHA](#)) is a speculative biotechnology stock aiming to potentially cure monogenic Central Nervous System (CNS) diseases via AAV-based gene therapies. The sector, poised for substantial growth, is anticipated to burgeon to a remarkable \$82.24 billion by 2032. Spearheading TSHA's endeavors is TSHA-102, a candidate formulated to combat Rett Syndrome. This initiative has garnered momentum courtesy of the FDA's Fast Track Designation, auguring well for its market trajectory. However, TSHA is not without its risks, attributed to financial qualms, potential regulatory encumbrances, and the quintessential market volatility endemic to the biotech sphere. Despite these impediments, I surmise that the prospective efficacy of TSHA-102 could allow TSHA to tap into a potentially sizeable market, ostensibly making it a "buy" at these levels. Still, I think it's worth noting TSHA's inherently speculative nature.

Business Overview

Taysha Gene Therapies operates within the niche and crucial gene therapy market, specifically targeting monogenic diseases affecting the CNS. The company develops and aims to commercialize adeno-associated virus (AAV)-based gene therapies, envisioned as a cornerstone in mitigating or potentially eradicating monogenic CNS diseases. Revenue generation hinges on the successful development, approval, and subsequent commercialization of these therapies, targeting both rare and large patient populations, thereby encompassing a broad market scope. TSHA and [UT Southwestern](#) are progressing with a portfolio of gene therapy candidates.

The success of TSHA, and consequently the revenue generation, heavily relies on the efficacy and market acceptance of the AAV-based gene therapies they develop. Gene therapy is a high-stakes, high-reward market. The trajectory of the [gene therapy market](#), with a projected leap from \$15.46 billion in 2022 to a staggering \$82.24 billion by 2032, unveils a decade brimming with financial promise and revolutionary medical advancements. This significant market expansion underscores a broader shift towards personalized medicine, leveraging genetic insights to combat previously insurmountable genetic disorders.

At Taysha, we aim to address this devastating need through our pipeline of AAV-based gene therapies.

Our gene therapy candidates are designed to target the unique, underlying biology of diseases of the CNS. We are focused on advancing our lead clinical program in Rett syndrome.



Source: Taysha's website

The high costs of certain gene therapies, sometimes soaring into [millions](#), pose a considerable barrier to market expansion despite the alleviation insurance coverage provides. These prices not only reflect production costs but also the groundbreaking value and extensive R&D behind these therapies. As the gene therapy field matures and becomes more competitive, prices may potentially become more accessible.

Currently, TSHA focuses on advancing TSHA-102 to treat Rett syndrome, a rare neurodevelopmental disorder triggered by mutations in the X-linked MECP2 gene. This disorder manifests through a range of challenges, including intellectual disabilities, communication loss, seizures, developmental regression or slowing, along with motor and respiratory impairments, consequently leading to a reduced life expectancy. The detrimental impact of Rett syndrome, stemming from a pathogenic or likely pathogenic MECP2 mutation, is prevalent, with an estimated 15,000 to 20,000 individuals affected across the United States, European Union, and the United Kingdom.

Pivotal Shifts and Promising Horizons

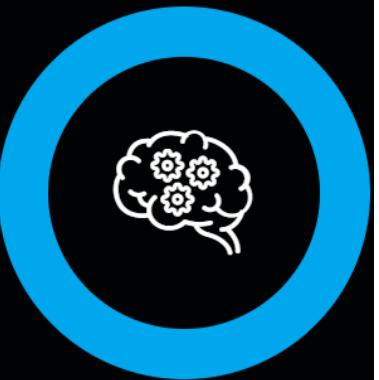
TSHA has recently experienced a gamut of developments, starting with the [halting](#) of its drug candidate TSHA-120's development due to challenges in crafting a supportive study for FDA market application. The drug was a therapy for giant axonal neuropathy (GAN). Despite this halt, TSHA is exploring external strategic channels for TSHA-120's further development. Interestingly, this cessation turned serendipitous, extending TSHA's financial solvency through Q4 2025 with a [\\$150 million](#) private placement, steering resources toward another promising venture, TSHA-102, for treating Rett syndrome.

Transitioning to a brighter spectrum, TSHA experienced a stock price uplift post the announcement of regaining compliance with certain [Nasdaq](#) stock market requirements regarding minimum market value and bid price. This compliance, confirmed by Nasdaq notifications, fueled an approximately 18% uptick in TSHA's shares, marking a pinnacle of intraday gains for the year. This positive market echo, following an earlier rise due to about a \$150 million private placement, not only underscores TSHA's robust market stance but also mirrors investor trust, substantiated by eight Buy ratings, a Hold rating, and zero Sell ratings.

Adding a milestone, TSHA clinched the FDA's [Fast Track Designation](#) for its gene therapy, TSHA-102, aimed at treating Rett syndrome, catalyzing a roughly 10% pre-market ascent in TSHA's stock. This designation is a stride towards expediting TSHA-102's journey through a Phase 1/2 trial in Canada towards FDA approval, especially when no FDA-approved treatments exist for the disorder it targets. This acceleration signifies not only a regulatory milestone but also a beacon of hope for early patient access to this treatment, with the roadmap to administer TSHA-102 to the first pediatric patient in Q1 2024, post-FDA clearance for a children's trial.

TSHA-102

TSHA-102 is a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a neurodevelopmental disorder and one of the most common genetic causes of severe intellectual disability, characterized by rapid developmental regression and in many cases caused by heterozygous loss of function mutations in MECP2, a gene essential for neuronal and synaptic function in the brain. TSHA-102 is constructed from a neuronal specific promoter, MeP426, coupled with the miniMECP2 transgene, a truncated version of MECP2, and miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel, packaged in self-complementary AAV9, which enables cellular regulation of both endogenous and exogenous MECP2 expression.

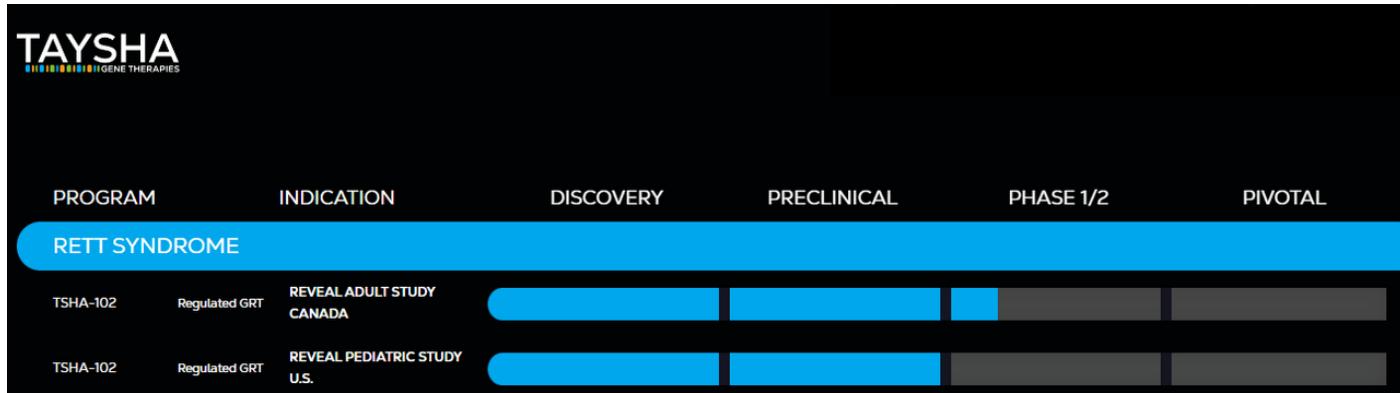


Source: Taysha's website

Moreover, TSHA unveiled encouraging preclinical in vitro data on TSHA-102 at the European Society of Gene & Cell Therapy 30th Annual [Congress](#). This data illustrated that the miRARE control element in TSHA-102 effectively regulates MECP2 transgene and protein expression in human and mouse cell lines, resonating with its potential to combat Rett syndrome. TSHA-102's unique miRNA-Responsive Auto-Regulatory Element (miRARE) technology aims to balance MECP2 levels in the CNS without overexpression risk. With clinical data from two adult patients dosed with TSHA-102 due for report in mid-November and plans to dose the first pediatric patient in Q1 2024, TSHA continues to weave a narrative of hope and potential in addressing monogenic CNS diseases.

Upcoming Report

TSHA is expected to report earnings on [November 14](#). Judging from the [latest developments](#), I'd argue a critical focus will be the clinical advancement of TSHA's leading candidate: TSHA-102. Data from the Phase 1/2 REVEAL trial concerning the dosing of the first adult patient with Rett Syndrome and the initial clinical safety findings will offer insights into TSHA-102's safety profile, a crucial aspect of its regulatory journey. The progression of events hinges on the outcomes of the ongoing Phase 1/2 clinical trials with adults and the initiation of clinical trials with children for TSHA. A key point in this scenario includes the feedback on the Clinical Trial Application (CTA) submitted to the UK's MHRA.



Source: Taysha's website

Furthermore, engagements with regulatory authorities, particularly the FDA, will be highlighted in the next earnings report. Key regulatory activities include submitting persuasive findings to the FDA, the awaited formal meeting in the third quarter regarding alternative study designs, and feedback on the CMC module 3 amendment submission, detailing commercial process product manufacturing and drug comparability analysis. Monitoring updates and further explanations on these regulatory interactions will elucidate the regulatory framework and potential hurdles or accelerants for TSHA's clinical programs.

Valuation Outlook

On the **financial** spectrum, the substantial cutback in R&D and G&A expenses last quarter—from \$38.2 million to \$12.5 million and \$11.5 million to \$8.8 million, respectively—is worth monitoring to see if this trend continues. Should this cost-management trajectory persist, it could bolster TSHA's financial posture, extending its runway to propel its clinical programs forward. But, such cuts could also hamper TSHA's R&D capabilities, so it's a double-edged sword.

Nevertheless, I believe TSHA's main value lies in its potential, particularly in its key revenue-generating project: the TSHA-102. This project leads TSHA's venture into treating Rett Syndrome, a rare genetic disorder. The ongoing REVEAL Phase 1/2 trial assesses the safety and initial efficacy of TSHA-102 in adult females with Rett syndrome. Early results are encouraging, with a single dose showing substantial improvements in clinical evaluations, autonomic functions, vocalization, and motor skills without major adverse incidents. However, TSHA-102's use as a one-dose treatment remains uncertain until further testing. For context, market forecasts predict growth in the Rett Syndrome sector, with projections estimating a rise from \$225.02 million in 2022 to \$515.06 million by 2029, at a [10.23% CAGR](#).

Yet, these figures don't account for gene therapies like TSHA-102. Accordingly, to gauge the revenue potential of TSHA-102, which would be the [first-ever](#) gene therapy for Rett Syndrome, consider this: Rett Syndrome affects [1 in 10,000 females](#). In a US market with 165 million females, that's potentially 16,500 patients for TSHA-102. While it's early to fix a price for TSHA-102, we can reference other gene therapies. For example, [Zolgensma](#) for spinal muscular atrophy costs \$2.1 million per single dose. [Luxturna](#), for a rare inherited blindness, costs \$425 thousand per eye. [Yescarta](#) and [Kymriah](#), which involve genetically modifying patient cells to treat certain cancers, range from \$373 thousand to \$475 thousand. Given these figures, I'd estimate that if approved, TSHA-102 would be priced at a minimum of \$373 thousand, aligning with the lower end of current gene therapy costs.

Therefore, the market could be worth between \$6.15 billion (at \$373 thousand per treatment) and \$34.65 billion (at \$2.1 million per treatment). While these numbers hint at TSHA-102's TAM, they are not concrete projections or price targets but rather an indication of its financial opportunity if successfully launched. Thus, considering TSHA's whole context, I think these factors make TSHA a good *speculative* buy at its current price.

Inherent Biotech Risks And Cash Runway

TSHA is developing gene therapy for specific brain diseases, a field with stringent regulations. Their product, TSHA-102, is still in its early Phase 1/2 testing stages. Achieving FDA approval is resource-intensive. A glance at [TSHA's financials](#) reveals a debt of \$60.4 million, a quarterly negative [free cash flow](#) of \$15.8 million, and cash reserves of \$45.1 million. When accounting for cash from operations and net CAPEX, the quarterly cash burn is \$18.8 million. This results in an annualized cash burn rate of \$63.2 million to \$75.2 million. With their current cash reserves of \$45.1 million, the projected cash runway is 0.60 to 0.71 years. Given this, TSHA may soon require additional financing, and if they choose equity financing, it could dilute value for existing shareholders.

Also, it's worth noting that TSHA's reliance on the efficacy of its AAV-based gene therapies is a risk that could be uncovered during clinical trials. Any adverse efficacy data or safety concerns could severely impede commercialization. Moreover, TSHA's strategy to serve rare and larger patient demographics could stretch resources thin, which is concerning given its dwindling cash reserves.



The market appears to be optimistic regarding TSHA's prospects. (TradingView)

Hence, as a whole, the blend of regulatory hurdles and gene therapy costs, alongside TSHA's financial constraints, accentuates TSHA's still challenging situation. Despite a seemingly cheap valuation, there's still a long road ahead until an ultimate FDA approval, which slightly tempers my optimism. Yet, once a drug receives [Fast Track Designation](#), the FDA usually communicates early and frequently throughout the entire drug development and review process, [which can expedite](#) TSHA-102's time to approval.

Conclusion

Overall, TSHA has a nuanced biotech investment profile. It's brimming with potential yet laden with substantial hurdles. Its prime candidate, TSHA-102 for Rett Syndrome, has garnered attention from the FDA's Fast Track Designation. However, the financial and regulatory challenges ahead are non-trivial, epitomizing the speculative nature inherent to TSHA. In my view, the potential efficacy of TSHA-102, juxtaposed with the forecasted growth in gene therapy and TSHA-102's market potential if approved, is why I rate TSHA a "buy." Nevertheless, the biotech sector's volatility, characterized by regulatory and market hurdles, mandates a prudent investment approach. While TSHA-102 could bolster TSHA's revenue, aligning with bullish market projections, the path is uncertain. It's imperative, I believe, for investors to proceed with a well-informed, cautious stance, carefully balancing optimism and due diligence.

This article was written by



Myriam Alvarez

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My name is Myriam Hernandez Alvarez. I received the Electronics and Telecommunication Engineering degree from the Escuela Politecnica Nacional, Quito, Ecuador, the M.Sc. degree in computer science from 

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Taysha Gene Therapies: What Could Possibly Go Wrong?

Jun. 14, 2021 2:25 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | 1 Comment | 5 Likes

**Marty Chilberg**

2.67K Followers

Summary

- Taysha Gene Therapies has gone from founding to an IPO in less than nine months.
- A unique collaboration with UTSW provided a deep pipeline of CNS programs with no future milestone or royalty obligations.
- Lead programs were acquired from Abeona and Hannah's Hope in the last year, accelerating the need for Taysha to become clinically ready.



dem10/iStock via Getty Images

Synopsis

Taysha (NASDAQ:[TSHA](#)) is a unique story in the gene therapy sector which makes them worth monitoring. This company, with an AveXis pedigree, is a spinoff, rollup and financial engineering concoction which begs the question: What could possibly go wrong?

This article is an introduction to Taysha, which closed two private investment rounds and an initial public offering just nine months after being founded in January 2020. With funding of \$300 million and 26 pipeline programs, they now are in the process of building the company. Taysha is worth watching, but investment is not advised given execution risks.

History

Taysha was founded on January 1, 2020, by former AveXis executives Sean Nolan and RA Session II. Prior to founding, Taysha signed an agreement with UT Southwestern or UTSW for 2.2 million shares, representing 20% ownership in the company. In exchange, Taysha received an initial pipeline of 15 AAV9 vector gene therapy programs plus ongoing discovery and preclinical research activities led by [Dr. Steven Gray](#) who was named Taysha's Chief Science Advisor.

In April 2020, Taysha received \$30 million in seed capital from Nolan Capital and PBM Capital. In August 2020, Taysha closed a \$95 million series B round from 13 investors including Venrock, Perceptive and Sands. Six weeks later, Taysha completed an initial public offering raising another \$150 million. In less than nine months, Taysha raised almost \$300 million with a couple dozen employees by leveraging UTSW's pipeline. The market valued this at over \$700 million.

As of December 31, 2020, Taysha had grown to [38 employees](#) with a pipeline of 25 programs – two of which were approved for the clinic. Two of the new programs were acquired from Abeona ([ABEO](#)). The first - [CLN1 had its origin at UTSW](#) before being licensed to Abeona. Taysha acquired the rights to this program in August 2020 for \$7 million plus potential future milestones of \$56 million and high single-digit royalties. Taysha added the Abeona Rett Syndrome program two months later for \$3 million with the same milestone and royalty structure. The other added programs came from UTSW.

The first quarter of [2021 updates](#) included:

- One new program added. Taysha acquired the rights to TSHA-120 from Hannah's Hope Fund for \$5.5 million plus potential milestones of \$19.3 million and low single-digit royalties.
- Cash dropped modestly by \$23 million to \$229 million.

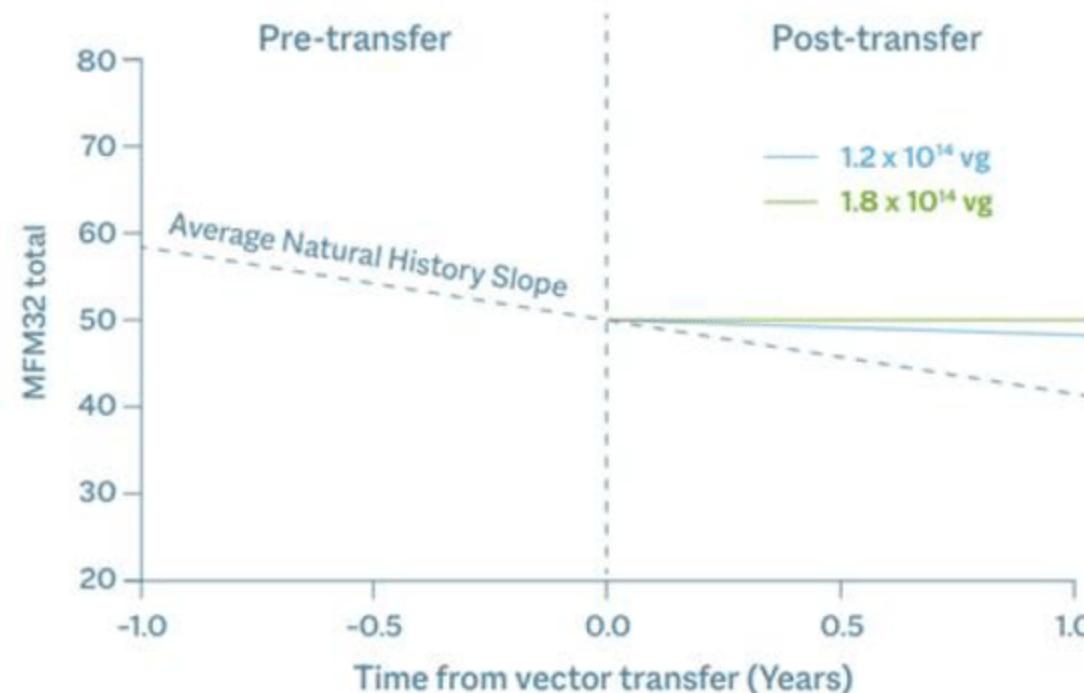
Pipeline

Taysha disclosed a pipeline totaling 26 CNS programs at the end of 1Q21. The three programs approved for the clinic were:

- **TSHA-101** AAV9 gene therapy for GM2 gangliosidosis (Tay-Sachs, Sandhoff disease). Phase I/II trial initiated in Canada by Queen's University. Safety and biomarker data expected by CYE21. FDA IND submission expected by CYE21.

- **TSHA-118** AAV9 gene therapy for CLN1 (Batten disease). IND is open. Expect to initiate trial by CYE21.
- **TSHA-120** (acquired from Hannah's Hope). This AAV9 gene therapy for Giant Axonal Neuropathy or GAN has orphan drug and rare pediatric disease designations. The P I/II clinical trial, conducted by the NIH, has dosed 14 patients intrathecally using four dose levels. Using Bayesian analysis, Taysha has concluded that treatment has a clinically meaningful slowing of the disease progression in a dose response relationship. The company intends to engage with regulatory agencies to discuss the pathway and will provide an update by year-end. The company has shared preclinical research (wild-type rats) that support redosing in the Vagus Nerve. This research indicates pre-existing neutralizing antibodies may dampen transduction, but efficient transduction of relevant neurons can still be achieved.

Bayesian Methodology



	Bayesian Analysis		Frequentist Analysis		
	Mean	Std Dev	Estimate	Std Error	p-Value
Post infusion: 1.8×10^{14} total vg	7.78	1.94	7.78	1.89	<0.001
Post infusion: 1.2×10^{14} total vg	6.09	2.11	6.07	2.05	0.004
Natural history decline	-8.19	0.74	-8.18	0.72	<0.001

Taysha 10-Q

The full pipeline consists of 26 programs including 14 in discovery, 9 in preclinical and 3 approved for human testing.



GRT: Gene replacement therapy; miRNA: microRNA; shRNA: short hairpin RNA.

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[Taysha website](#)

The UTSW collaboration has an enviable list of pipeline programs which undoubtedly helped Taysha capitalize the company. But how many will eventually make it to the clinic or become commercially available? And why so many discovery stage programs? Those questions could be linked to a high failure rate for all preclinical programs, but also could be tied to a desire to acquire funding for the university research. The UTSW program has relied on family-funded research which some [medical ethicists](#) see as problematic. The agreement stipulates that Taysha may terminate programs or indications after the initial research. Taysha is required to use reasonable efforts to develop, obtain regulatory approval for and *commercialize at least one licensed product*.

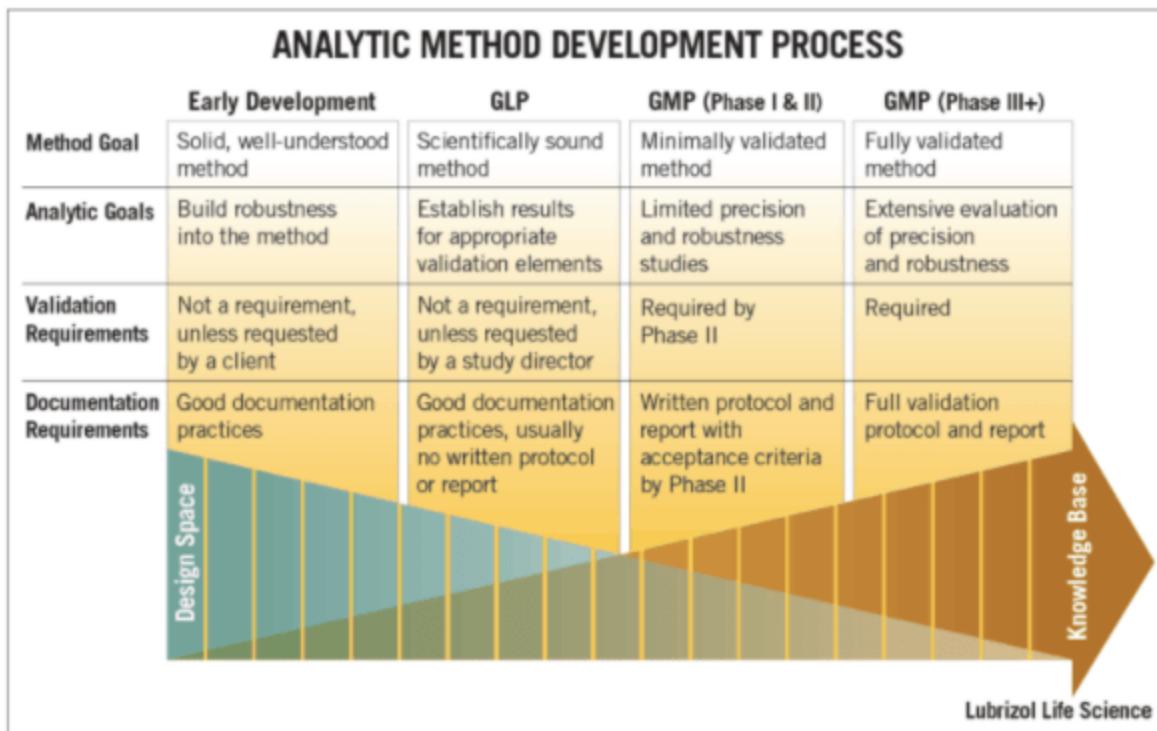
Summary - Now What?

The assembly of Taysha Gene Therapies resembles a Lego set. The pieces supplied by UTSW were acquired for 20% ownership. PBM handled outsourced administration activities to initially operate the company as the fund raising commenced. CEO RA Session II utilized his financial expertise and that of private equity firms Nolan Capital and PBM Capital to rapidly monetize the deal with a series of funding events.

A replenishing pipeline of discovery and preclinical programs is excellent in theory – especially when no future milestone or royalty obligations exist. Mix in two acquired programs from Abeona and one from Hannah's Hope and programs approved for the clinic are stapled to the pipeline. The challenges now begin as programs transition to Taysha, who is responsible for all clinical development, regulatory and commercialization activities.

A significant priority is staffing. Remember that this organization had no employees 1.5 years ago and only 38 five months ago. Now LinkedIn shows 98 employees (not always accurate). The [careers section](#) on Taysha website shows 40 open jobs.

Staffing alone will not solve the [bioanalysis](#) requirements. Biomarkers and assays take time to develop and validate. Analytical Method Development requirements evolve as programs progress.



[Lubrizol Life Science](#)

The company is following the AveXis roadmap with respect to in-house manufacturing. They announced a plan to invest \$75 million in Durham for preclinical, clinical and commercial gene therapy production. Added to preclinical production at UTSW and Catalent outsourced manufacturing, it provides a three-pronged production solution... and one that may be valuable to potential acquirers as was seen when AveXis was acquired.

Mix in additional research programs including redosing, miRNA gene expression and directed evolution, and it adds concerns that Taysha is maybe over-reaching.

Since the discovery of miRNAs in the earlier 1990s, tremendous progress has been made on how miRNAs are produced within cells, how they exert regulatory effects on gene expression, and how they are involved in various physiological and pathological events. It is now clear that miRNAs are powerful gene regulators, and that they not only help control mRNA stability and translation but are also involved in transcription. However, our understanding of when and how miRNAs can exert regulatory effects on transcription is limited. Similarly, the conditions under which miRNAs elicit translational activation need to be further explored.

Publication

Taysha is a company worth watching but likely is not investment worthy until more of the organization is built and functional. By then we may also have answers about pipeline viability.

This article was written by



Marty Chilberg

2.67K Followers

Marty Chilberg is a seasoned financial professional with over 30 years of C-Suite, board, consulting and advisory experience. He began his career as a certified public accountant (CPA). He moved to Silicon Valley in

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Home > ETFs and Funds Analysis > Closed End Funds Analysis

BMEZ: Attractive Discount In This Beaten Down Name

Jun. 13, 2022 3:07 AM ET | **BlackRock Health Sciences Term Trust (BMEZ)** | BIGZ, BSTZ, DGX... |
22 Comments | 17 Likes



Nick Ackerman

Investing Group Leader

Summary

- BMEZ has been getting hit significantly, with the biotech and growth space moving sharply lower.
- Despite these pressures, I believe that BMEZ is attractive given its current discount and selloff.
- With our recent update on BlackRock Science & Technology Trust II, the fund's distribution is hanging on for now, but we shouldn't be surprised to see a cut before year-end.
- I am Nick Ackerman, a former financial advisor with 15+ years experience. I contribute to the investing group [CEF/ETF Income Laboratory](#) where I share exclusive high-yield opportunities in the CEF/ETF space.



adventtr/E+ via Getty Images

Written by Nick Ackerman, co-produced by Stanford Chemist. This article was originally published to members of the CEF/ETF Income Laboratory on June 10th, 2022.

With [our recent update](#) on BlackRock Science & Technology Trust II ([BSTZ](#)), it only seems appropriate to do an update for the sister fund, BlackRock Health Sciences Trust II (NYSE:[BMEZ](#)). BMEZ is struggling for much the similar reasons that BSTZ is. However, it has a healthcare focus, and the biotech sector has been taking a hit. As the biotech name would suggest, it's an area of technology. Since that means growth, we've seen the fund tumbling. Nobody wants growth investments at this time while rates are rising, especially [when the latest inflation](#) number came in hot once again.

That being said, I think that we are looking at a much more attractive price for BMEZ. Combining that with the fund's discount being quite attractive, I think BMEZ is a fair option to consider picking up at these levels. Similar to BSTZ, I'm not sure when a rebound will happen, but it could prove rewarding if you can handle higher risks in the long term. This is a change from my previous update: I liked the fund, but was sticking with a "Hold" rating. I'll be flipping this to a "Buy" rating now. That doesn't mean it will perform well soon, of course. It reflects that I believe there is a greater than 50% chance it will be higher in the next year or two.

The Basics

- 1-Year Z-score: -1.25
- Discount: 12.62%
- Distribution Yield: 10.39%
- Expense Ratio: 1.3%
- Leverage: N/A
- Managed Assets: \$2.149 billion
- Structure: Term (anticipated liquidation January 29th, 2032)

BMEZ "[seeks](#) to invest up to 25% in private companies." It intends to do this through "at least 80% of its total assets in equity securities of companies principally engaged in the health sciences group of industries and equity derivatives with exposure to the health sciences group of industries." With this, it also utilizes an options strategy.

It was last [reported](#) that 24.14% of the portfolio was overwritten. This is below its target range of 30% to 40% and would indicate a bullish stance. In hindsight, if they were more aggressive in this strategy, it could have offset a bit more of the losses.

At this point, keeping a lower percentage overwritten seems appropriate, so positions aren't called away during a rebound. Of course, that is whenever such a rebound might happen. It could take a while to get through the pressures of higher interest rates and see through to the other side when inflation starts to cool off.

The fund has a [term structure](#) that will see the fund potentially liquidated around Jan. 29, 2032. They may switch to a perpetual fund after a tender offer for 100% of outstanding shares at 100% of NAV. If there are still \$200 million in total net assets, the board can convert to a perpetual structure. After that point, there will be no more support to keep the fund to its NAV.

Of course, it isn't realistic to believe there won't be a change in the fund's price and NAV over the next ten years. It's something to continue to monitor and can be taken advantage of closer to the fund's termination date. Ideally, we'd see the NAV and price rise from current levels.

The term structure keeps the fund from trading at a perpetual discount. If the fund performs well, the fund will likely continue to operate. After the fund's launch, it made some significant moves that certainly helped give it a jump start towards that goal.

Performance - Attractive Discount

One interesting note is that BMEZ is now larger than BSTZ. These are both funds of significant size, as BlackRock ([BLK](#)) tends to produce large funds. Thanks to their massive size, they have many channels to sell through. This change was the result of BMEZ holding up relatively better than BSTZ. While BMEZ still has private holdings in smaller, more biotech-related companies, they still have some exposure to traditional healthcare too. That seems to have provided a bit of shelter, relatively speaking.

Below we are seeing the YTD results between BMEZ and BSTZ (just for fun, as it isn't comparable) and the SPDR S&P Biotech ETF ([XBI](#)). As we can see, BMEZ is holding up significantly better than if it was a pure-play biotech fund. This is to be expected and helps highlight that despite the wreckage we are seeing, it isn't all bad news.

- BMEZ Total Return Price % Change
- XBI Total Return Price % Change
- BSTZ Total Return Price % Change



- BMEZ Total Return NAV % Change
- XBI Total Return NAV % Change
- BSTZ Total Return NAV % Change

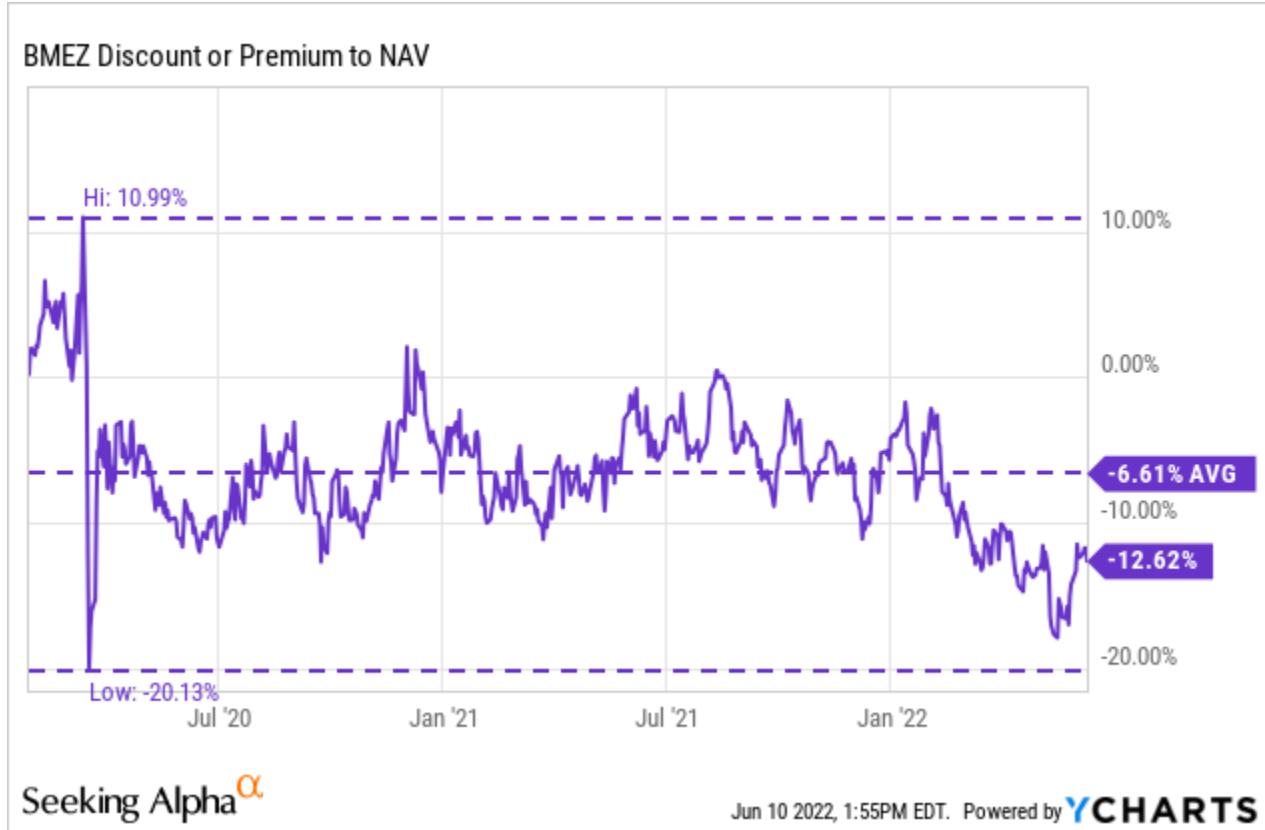


Seeking Alpha^α

Jun 10 2022, 1:53PM EDT. Powered by **YCHARTS**

Ycharts

The comparison between the total share price and total NAV return has also produced a widening discount for the year. We aren't exactly at the widest discount yet, but meaningfully below the average discount. It could still be seen as a relatively new fund, so history is rather short.

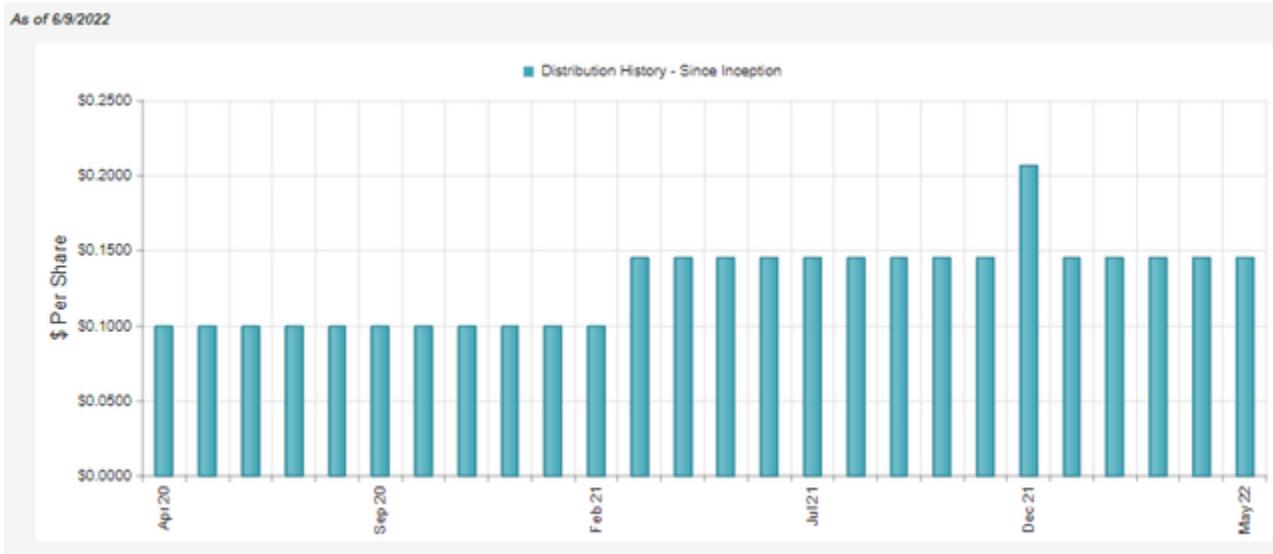


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Distribution - Juicy Yield, But Very Cautious

One thing that we mentioned for BSTZ is applicable here too. We noted that BlackRock just made several adjustments to their monthly distributions for CEFs. However, they held BSTZ alone, and they also left BMEZ alone. Yet they got the other sister fund, BlackRock Innovation and Growth Trust ([BIGZ](#)).

BlackRock announces distributions every month, so cuts can be announced anytime. However, since they made several adjustments already, I'm leaning towards them holding out for the next quarter to see if there is a rebound.



BMEZ Distribution History (CEFConnect)

When looking at coverage for BMEZ's distribution, it will come entirely from capital gains. The fund provides no actual net investment income. This is simply because the underlying holdings do not pay sufficient amounts of dividends or interest to offset the earnings. Total investment income came to less than \$6.7 million. For a fund that ended 2021 with almost \$3 billion in assets, that's certainly not a big driver of the fund.

Therefore, we see net investment losses rather than NII.

	BMEZ	
	Year Ended 12/31/21	Period from 01/30/20 ^(a) to 12/31/20
INCREASE (DECREASE) IN NET ASSETS		
OPERATIONS		
Net investment loss	\$ (36,221,355)	\$ (24,489,080)
Net realized gain (loss)	370,126,396	131,179,423
Net change in unrealized appreciation (depreciation)	<u>(621,751,428)</u>	<u>1,203,954,044</u>
Net increase (decrease) in net assets resulting from operations	<u>(287,846,387)</u>	<u>1,310,644,387</u>
DISTRIBUTIONS TO SHAREHOLDERS^(b)		
From net realized gain	(192,905,388)	(101,402,843)
Return of capital	—	—
Decrease in net assets resulting from distributions to shareholders	<u>(192,905,388)</u>	<u>(101,402,843)</u>
CAPITAL SHARE TRANSACTIONS		
Net proceeds from the issuance of shares	—	2,253,296,500
Reinvestment of distributions	—	—
Redemption of shares resulting from share repurchase program (including transaction costs)	—	—
Net increase in net assets derived from capital share transactions	<u>—</u>	<u>2,253,296,500</u>
NET ASSETS		
Total increase (decrease) in net assets	(480,751,775)	3,462,538,044
Beginning of period	<u>3,462,638,044</u>	<u>100,000</u>
End of period	<u>\$ 2,981,886,269</u>	<u>\$ 3,462,638,044</u>

BMEZ Annual Report (BlackRock)

With their options strategy, they could be generating some gains through that route this year. However, in prior years, this actually resulted in losses for the fund. For fiscal 2021, it wasn't meaningful losses, but they were losses nonetheless. This can result from the fund closing out options positions at a loss to not have the underlying name called away.

	BGY	BME	BMEZ	BIGZ ^(a)
INVESTMENT INCOME				
Dividends — unaffiliated	\$ 17,372,777	\$ 6,458,167	\$ 6,794,808	\$ 8,664,340
Dividends — affiliated	782	1,532	5,437	42,953
Securities lending income — affiliated — net	—	21,952	276,651	118,573
Foreign taxes withheld	(1,085,632)	(79,721)	(393,701)	—
Total investment income	<u>16,287,927</u>	<u>6,401,930</u>	<u>6,683,195</u>	<u>8,825,866</u>
EXPENSES				
Investment advisory	7,005,794	5,898,369	41,309,410	43,612,551
Trustees and Officer	158,682	40,031	194,704	171,333
Custodian	127,533	80,407	280,446	157,631
Professional	98,220	97,125	216,639	428,373
Transfer agent	88,211	67,679	125,261	128,999
Accounting services	48,758	28,854	326,183	315,288
Registration	36,936	11,658	—	—
Offering	—	61,531	—	—
Miscellaneous	59,887	67,435	467,025	109,900
Total expenses excluding excise tax	<u>7,624,021</u>	<u>6,353,089</u>	<u>42,919,668</u>	<u>44,924,075</u>
Excise Tax	—	12,114	—	—
Total expenses	<u>7,624,021</u>	<u>6,365,203</u>	<u>42,919,668</u>	<u>44,924,075</u>
Less:				
Fees waived and/or reimbursed by the Manager	(702,840)	(5,096)	(15,118)	(362,833)
Total expenses after fees waived and/or reimbursed	<u>6,921,181</u>	<u>6,360,107</u>	<u>42,904,550</u>	<u>44,561,242</u>
Net investment income (loss)	<u>9,366,746</u>	<u>41,823</u>	<u>(36,221,355)</u>	<u>(35,735,376)</u>
REALIZED AND UNREALIZED GAIN (LOSS)				
Net realized gain (loss) from:				
Investments — unaffiliated	61,890,631 ^(b)	54,102,268	374,700,990	(197,928,462)
Investments — affiliated	—	(67)	(851)	11,860,850
Foreign currency transactions	(381,094)	(4,943)	(61,561)	497,260
Options written	(16,019,721)	(7,325,999)	(4,512,182)	(14,153,519)
	<u>45,489,816</u>	<u>46,771,259</u>	<u>370,126,396</u>	<u>(199,723,871)</u>

BMEZ Annual Report (BlackRock)

For tax purposes, despite the lack of income generated from the fund, it gets taxed primarily at ordinary income rates. At least for the last two years. I would suspect that over the longer term, we will see long-term capital gains as the primary tax characterization of the distribution. We are also likely to see return of capital this year or next due to the losses that could be being realized through this year. We will get a better idea of this with the next Semi-Annual Report.

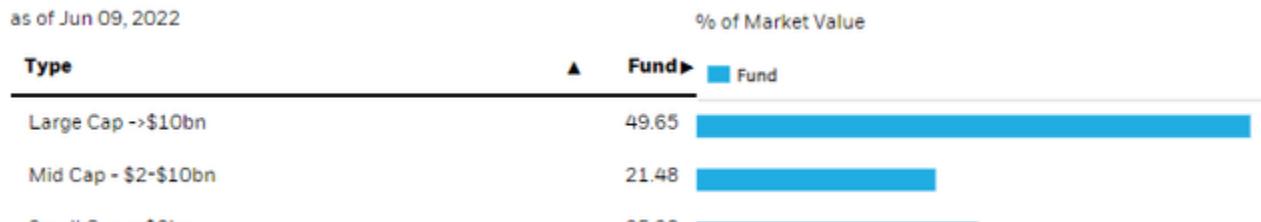
BMEZ		
Ordinary income	\$ 111,413,529	\$ 101,402,843
Long-term capital gains	81,491,859	—
	<u>\$ 192,905,388</u>	<u>\$ 101,402,843</u>

BMEZ Annual Report (BlackRock)

The reason we see it classified as ordinary income would seemingly come from short-term capital gains that are lumped in with ordinary income.

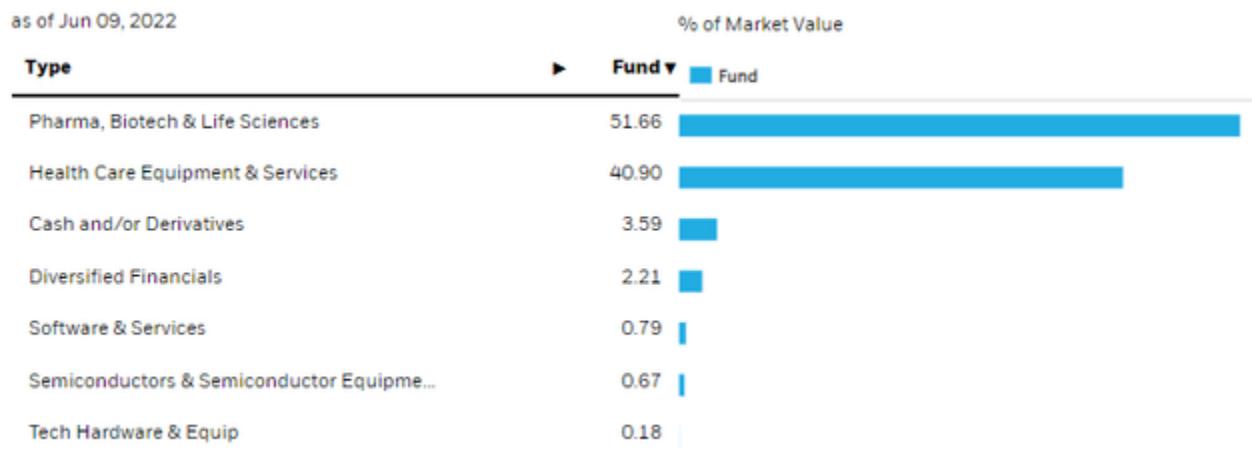
BMEZ's Portfolio

BMEZ is similar to BSTZ and BIGZ because they have a sleeve of private investments. They target up to 25% in private securities. However, they aren't limited to any specific geography or market cap. This fund favors U.S. investments, with its ~76% allocation in U.S.-based names. With private investments generally being smaller companies, we see some exposure to small and mid-cap names.



BMEZ Market Cap Weighting (BlackRock)

Where they are restricted or focused, though, is the healthcare space. When looking at the industry exposure, we see an overwhelming allocation to the top two healthcare-related spaces.



BMEZ Industry Weighting (BlackRock)

The weightings here don't change drastically. Since the last time we looked at the fund towards the end of January 2022, there haven't been any meaningful shifts.

In their private investments, biotech companies were the majority of these deals.

Private investments in BMEZ:³

Company	GICS Industry	Investment Quarter	Investment Amount (\$m)	Current Phase	Portfolio Ownership
Oak Street	Healthcare Providers & Services	Q1 2020	\$97.4	Public	Exited
Kymera Therapeutics	Biotechnology	Q1 2020	\$23.5	Public	Held
Sigilon Therapeutics	Biotechnology	Q1 2020	\$3.0	Public	Held
Annexon	Biotechnology	Q2 2020	\$15.3	Public	Held
Taysha Gene Therapies	Biotechnology	Q3 2020	\$4.0	Public	Exited
Everest Medicine	Pharmaceuticals	Q2 2020	\$21.1	Public	Held
Goldfinch	Biotechnology	Q2 2020	\$3.0	Private	Held
Insitro	Pharmaceuticals	Q2 2020	\$8.0	Private	Held
Antengene	Pharmaceuticals	Q3 2020	\$7.1	Public	Held
Kronos Bio, Inc.	Biotechnology	Q3 2020	\$4.6	Public	Held
Talaris Therapeutics	Biotechnology	Q3 2020	\$7.4	Public	Held
Olema	Biotechnology	Q3 2020	\$2.9	Public	Exited
LianBio	Biotechnology	Q4 2020	\$6.0	Public	Held
Decibel Therapeutics	Biotechnology	Q4 2020	\$3.9	Public	Held
Ambrx, Inc.	Biotechnology	Q4 2020	\$14.6	Public	Held
Imago Biosciences, Inc.	Biotechnology	Q4 2020	\$10.0	Public	Held
Acumen Pharmaceuticals, Inc.	Biotechnology	Q4 2020	\$3.3	Public	Held
Everly Well, Inc.	Healthcare Providers & Services	Q4 2020	\$10.0	Private	Held
Connect Biopharmaceuticals	Biotechnology	Q4 2020	\$14.6	Public	Held
Neurogene	Biotechnology	Q4 2020	\$9.6	Private	Held
Immuneering	Biotechnology	Q4 2020	\$6.0	Public	Held
Immunocore	Biotechnology	Q4 2020	\$5.9	Public	Held
Abbisko	Biotechnology	Q4 2020	\$14.8	Public	Held
Adicon	Healthcare Providers & Services	Q4 2020	\$17.8	Private	Held
IsoPlexis Corp	Biotechnology	Q4 2020	\$14.8	Public	Held
Affinivax, Inc.	Biotechnology	Q1 2021	\$5.8	Private	Held
TScan Therapeutics	Biotechnology	Q1 2021	\$10.0	Public	Held
Cellarity, Inc.	Biotechnology	Q1 2021	\$14.6	Private	Held
Design Therapeutics	Biotechnology	Q1 2021	\$10.0	Public	Held
Vividion Therapeutics	Pharmaceuticals	Q1 2021	\$8.0	Public	Held
Amunix, Inc	Biotechnology	Q1 2021	\$10.0	Acquired	Held
OnKure, Inc.	Biotechnology	Q1 2021	\$2.3	Private	Held
Omega Therapeutics	Biotechnology	Q1 2021	\$8.0	Public	Held
Monte Rosa	Biotechnology	Q1 2021	\$8.0	Public	Held
Rapid Micro Biosystems	Biotechnology	Q1 2021	\$9.9	Public	Held
Nucleix Limited	Medical Devices & Supplies	Q1 2021	\$6.9	Private	Held
Numab Therapeutics	Biotechnology	Q2 2021	\$7.6	Private	Held
Bright Peak Therapeutics	Biotechnology	Q2 2021	\$8.0	Private	Held
Nikang Therapeutics	Biotechnology	Q2 2021	\$6.0	Private	Held
PSI Quantum	Semiconductors	Q2 2021	\$15.0	Private	Held
Quanta Dialysis Tech	Healthcare Providers & Services	Q2 2021	\$22.0	Private	Held
eXo Imaging, Inc.	Medical Devices & Supplies	Q2 2021	\$13.2	Private	Held
Carbon Health Technology	Healthcare Providers & Services	Q3 2021	\$17.1	Private	Held
Laronde, Inc	Biotechnology	Q3 2021	\$13.5	Private	Held
Swift Health Systems	Medical Devices & Supplies	Q3 2021	\$6.4	Private	Held
Mirvie, Inc	Biotechnology	Q4 2021	\$8.0	Private	Held

BMEZ Private Holdings (BlackRock)

We can see that they have exited only three of these positions, all three of which have become publicly traded during the time they've held them. That would be Oak Street ([OSH](#)), Taysha Gene Therapies ([TSHA](#)) and Olema ([OLMA](#)). TSHA and OLMA being biotech companies, can really highlight just how much of a thrashing this space has received.

They are down to penny stock territory with a \$4.01 price on OLMA and TSHA at \$3.32.



Ycharts

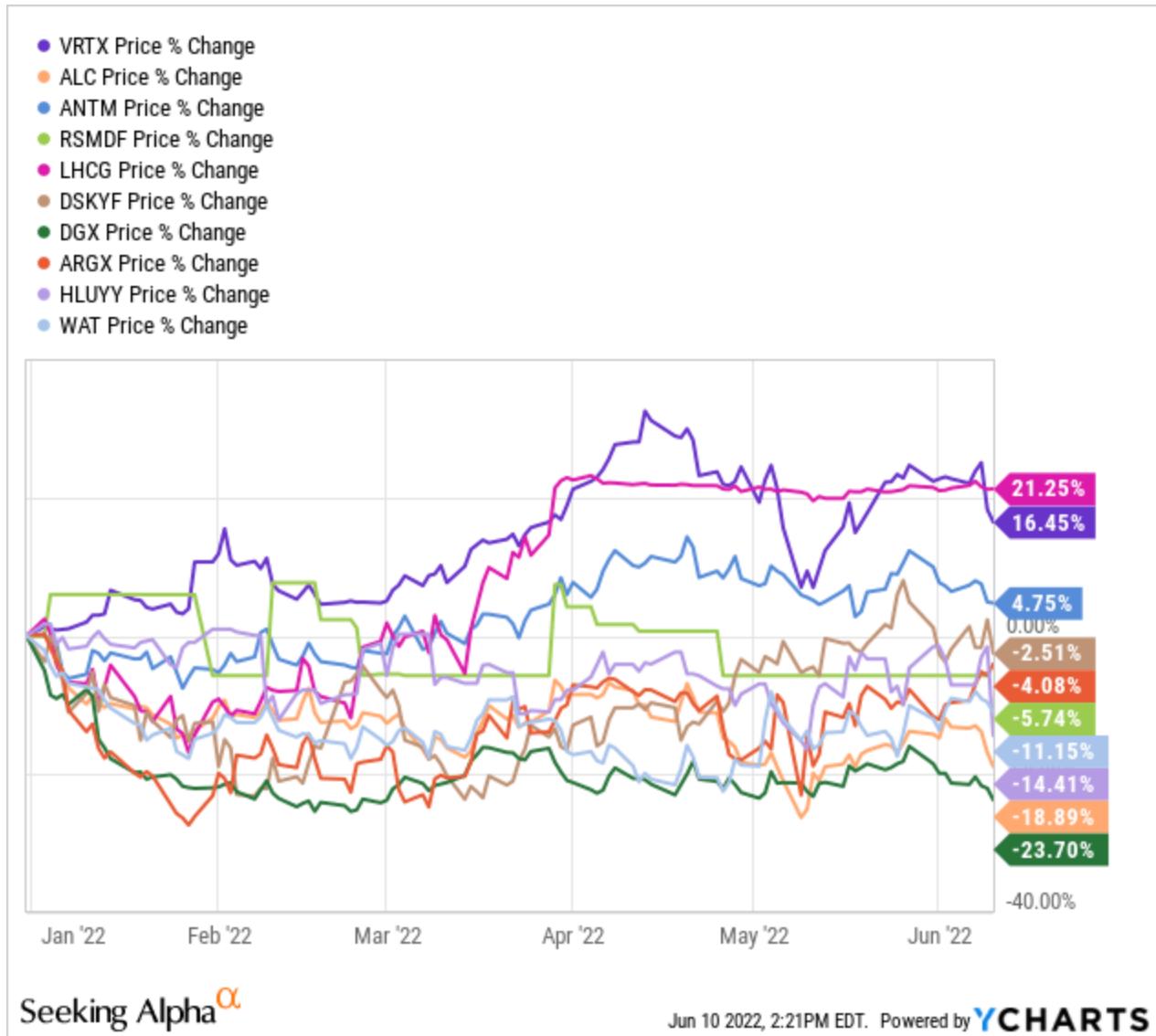
The current top ten names are showing a bit more promise, even though some of these are biotech names too.

as of Jun 09, 2022

Name	Weight (%)	Name	Weight (%)
VERTEX PHARMACEUTICALS INCORPORATED	2.81	DAIICHI SANKYO CO LTD	1.76
ALCON AG	2.45	QUEST DIAGNOSTICS INCORPORATED	1.61
ANTHEM INC	2.13	ARGENX SE	1.58
RESMED INC	1.88	H LUNDBECK A/S	1.55
LHC GROUP INC	1.78	WATERS CORPORATION	1.53

BMEZ Top Ten (BlackRock)

They are all publicly traded, too. This is interesting when you consider that BSTZ has a handful of its largest holdings as the private positions.



Ycharts

Quest Diagnostics (**DGX**) is performing the worst of this group of names. They are a healthcare services stock that provides diagnostic information and services - as the name would suggest.

Conclusion

Personally, nothing in this portfolio is anything I'd hold individual or even generally run across in my normal investing process. That's part of why I am comfortable with my BMEZ position to add diversification to my portfolio. While growth is out of favor, I don't mind being patient for an eventual rebound. The distribution is likely in danger at current levels unless we see a quick turnaround. However, it is expected to pay something at least while waiting. The latest discount and selloff, I believe, are making this a more attractive holding overall and worth adding to at this time. That is, if one is comfortable with exposure to a riskier area of the market.

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This article was written by



Nick Ackerman

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Nick Ackerman is a former financial advisor using his experience to provide coverage on closed-end funds and exchange-traded funds. Nick has previously held Series 7 and Series 66 licenses and has been investing personally for over 14 years.

He contributes to the investing group [CEF/ETF Income Laboratory](#) along with leader Stanford Chemist, and Juan de la Hoz and Dividend Seeker. They help members benefit from income and arbitrage strategies in CEFs and ETFs by providing expert-level research. The service includes: managed portfolios targeting safe 8%+ yields,

actionable income and arbitrage recommendations, in-depth analysis of CEFs and ETFs, and a friendly community of over a thousand members looking for the best income ideas. These are geared towards both active and passive investors. The vast majority of their holdings are also monthly-payers, which is great for faster compounding as well as smoothing income streams. [Learn more.](#)

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Home > ETFs and Funds Analysis > Closed End Funds Analysis

BMEZ: Buy Low, Hold For The Distribution And Rebound

Feb. 07, 2022 6:21 AM ET | **BlackRock Health Sciences Term Trust (BMEZ)** | ALC, ANNX, BME... |
56 Comments | 27 Likes



Nick Ackerman

Investing Group Leader

Summary

- BMEZ was one of the best performing funds in 2020, the year it launched as it hit the market at the right time.
- This success did not carry over to 2021, as it was one of the seemingly few funds to produce losses for the year.
- It has been an even tougher go as we enter into 2022, and the innovation/growth space goes into meltdown.
- Previously, we looked at BIGZ, BST and BSTZ, which are all related by being BlackRock funds and being invested in some of the worst-performing assets as of late.
- I am Nick Ackerman, a former financial advisor with 15+ years experience. I contribute to the investing group [CEF/ETF Income Laboratory](#) where I share exclusive high-yield opportunities in the CEF/ETF space.



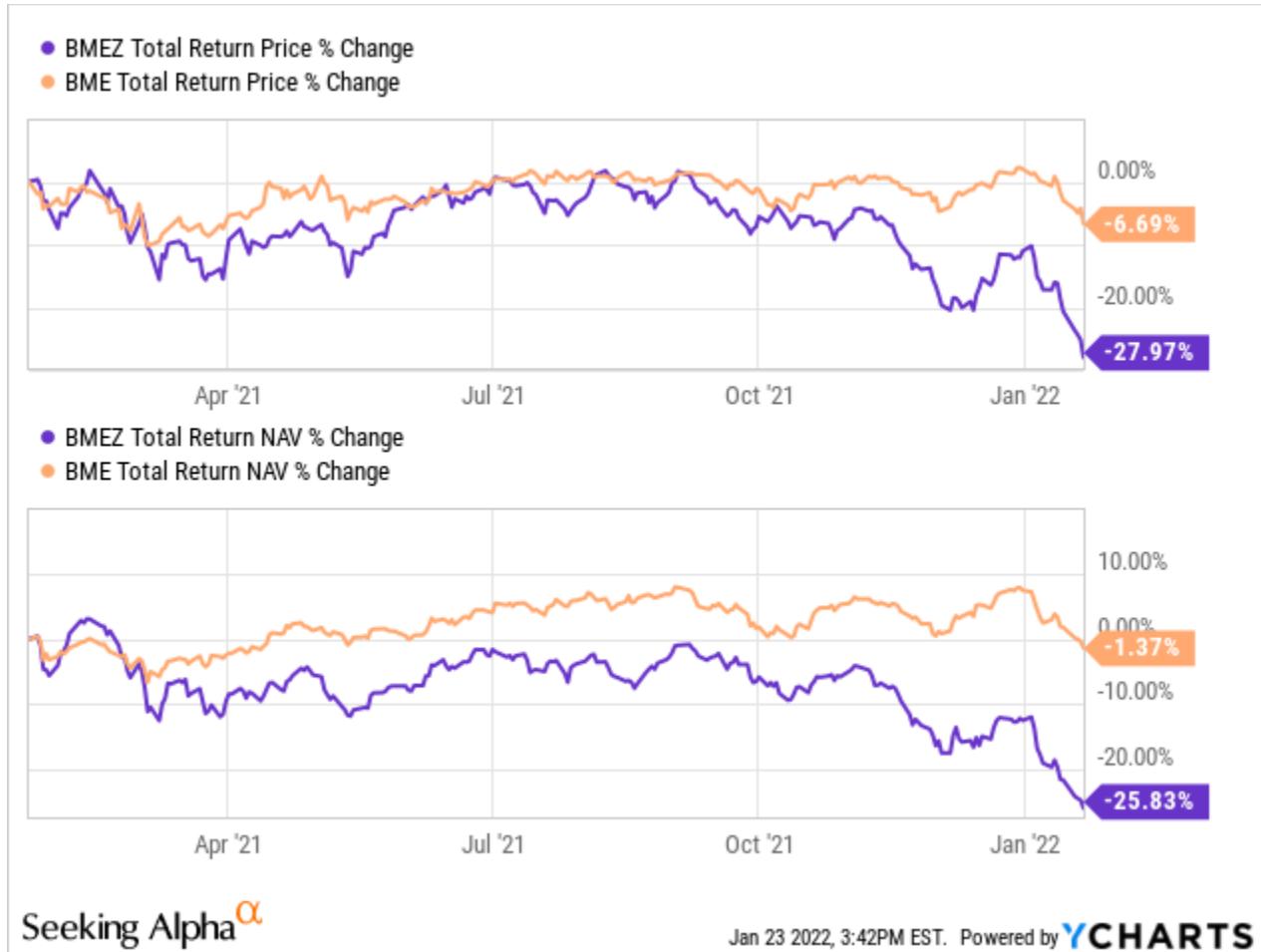
knape/E+ via Getty Images

Written by Nick Ackerman, co-produced by Stanford Chemist

BlackRock Health Sciences Trust II (NYSE:[BMEZ](#)) has been having a tough time performing. That is despite its exposure to healthcare as it is invested heavier in the healthcare innovation/growth space. It also has private holdings that also can increase its risk level. The losses had been accelerating as of late in its underlying holdings but I view it as being more attractively priced.

Over the last year, the fund has been down nearly 26% on a total NAV return basis. The losses accelerated significantly even more recently, with the broader markets also participating.

Over the last few months, it was mostly the innovation/tech space that was difficult. Below we can see the comparison between BMEZ and its predecessor, BlackRock Health Sciences Trust ([BME](#)). BME holds the more traditional healthcare names. We can see how much better BME has been holding up relative to BMEZ. However, losses have still occurred there too.

Seeking Alpha^α

Jan 23 2022, 3:42PM EST. Powered by YCHARTS

Ycharts

BMEZ's discount has been widening, and the overall decline could provide a fairly attractive entry into this fund. That being said, it remains a riskier holding and not for conservative investors. After the poor performance in 2021, it isn't exactly going to be the most encouraging investment either, which means that the discount could linger for quite some time. There weren't a lot of places that provided for losses in 2021, but BMEZ was one.

The Basics

- 1-Year Z-score: 0.85
- Discount: 3.55%
- Distribution Yield: 8%
- Expense Ratio: 1.31%

- Leverage: N/A
- Managed Assets: \$2.511 billion
- Structure: Term (anticipated liquidation January 29th, 2032)

BMEZ "seeks to invest up to 25% in private companies." It intends on doing this through "at least 80% of its total assets in equity securities of companies principally engaged in the health sciences group of industries and equity derivatives with exposure to the health sciences group of industries." With this, it also utilizes an options strategy.

It last reported 21.92% of the portfolio being overwritten. This is below its target range of 30% to 40% and would indicate a bullish stance. In hindsight, if they were more aggressive in this strategy, it could have offset a bit more of the losses. At this point, keeping a lower percentage overwritten seems appropriate, so positions aren't called away during a rebound. Of course, that is if there is a rebound and to how aggressive the rebound might be.

The fund has a [term structure](#) that will see the fund potentially liquidated around Jan. 29, 2032. They may switch to a perpetual fund after a tender offer for 100% of outstanding shares at 100% of NAV. If there are still \$200 million in total net assets, the board can convert to a perpetual structure. After that point, there will be no more support to keep the fund to its NAV.

Even if the fund finished flat from here, at least the 8.43% discount could be harvested. It isn't realistic to believe that there won't be a change in the fund's price and NAV over the next ten years. It's something to continue to monitor and can be taken advantage of closer to the fund's termination date.

The term structure is designed to keep the fund from trading at a perpetual discount. If the fund performs well, the fund would likely continue to operate. After the fund's launch, it made some significant moves that certainly helped give it a jump start towards that goal.

Performance - Disappointing So Far

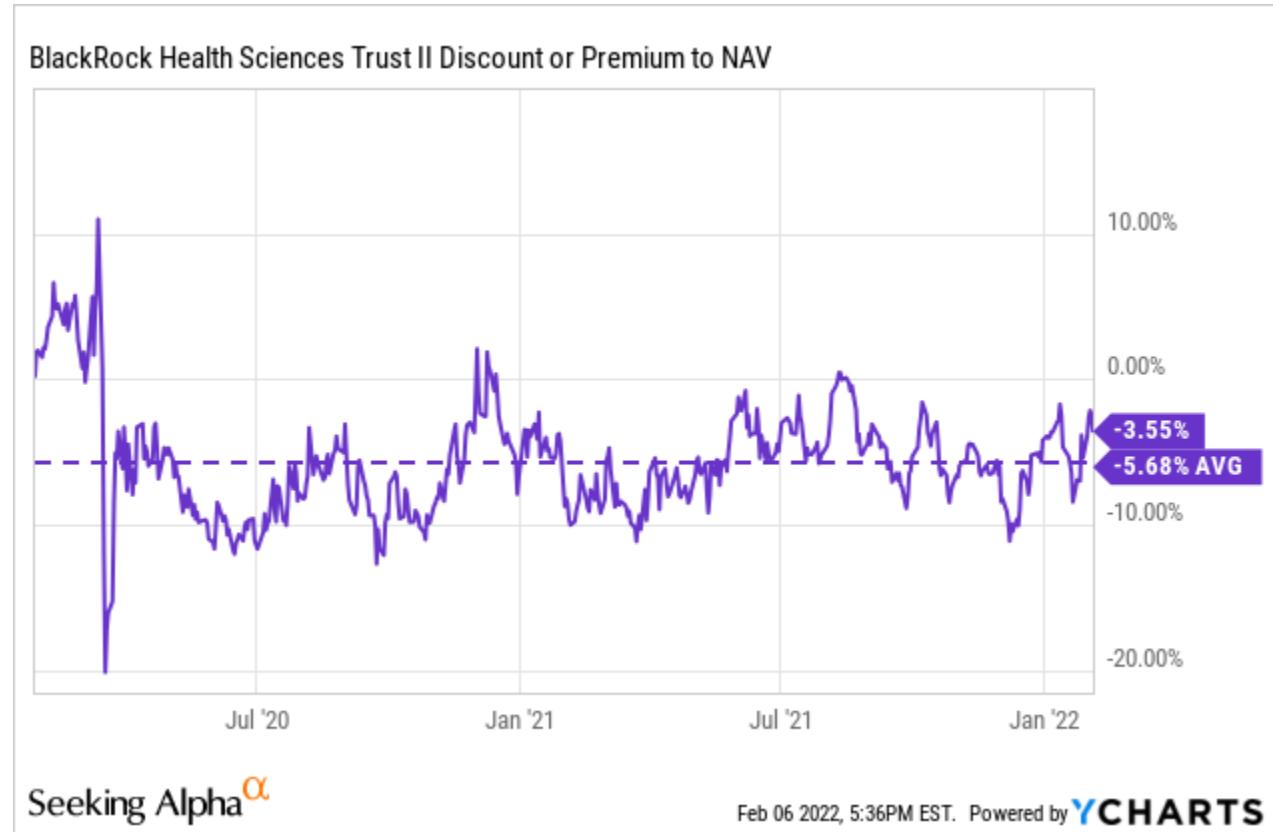
The fund seemed as though it had a lot of potentials when it first began. It was one of the best-performing funds in 2020. Although CEFs are just a function of their underlying investments - they are a wrapper for investments and not an asset class themselves.

The inception date of January 29th, 2020, just happened to be a great time to launch a fund of this type. The COVID pandemic hit, and they could take advantage of depressed prices to build out their portfolio. In addition to that, we also know that the innovation space was exactly what worked in 2020.

Since then, it has been a slow grind lower. 2021 delivered a total return of -8.31% and a total NAV return of -5.76%.

With the losses accelerated into 2022, BMEZ wouldn't necessarily look like a promising investment. However, I would take the other side and say that now is an even better time to invest. I'm not sure when the sell-off will let up or if we are just getting started with it. What I do know is that the fund's discount is looking fairly attractive, and the sell-off has definitely made it a lot cheaper than it had been.

Launching in 2020 means, we only have a couple of years' worth of data to look back on. That being said, we see that the fund's discount is around its average. It snapped back quickly from the latest dip. Still, the overall dip is presenting an attractive opportunity on its own.



Data by [YCharts](#)

CEFs tend to sell off harder than other investments due to their discount/premium mechanics. They are also owned mainly by retail investors, so they generally shoot first and ask questions later. That dries up the buyers for these funds, which can fall significantly below their NAV levels. For those of us that invest in CEFs, we generally see this as an opportunity to exploit.

Distribution - 8% Is Attractive, But Be Cautious

While the fund is pretty attractively valued now - a higher distribution rate is appealing - I'd have to be a bit somber here. It will not be sustainable if we don't start getting a rebound.

Currently, the distribution rate comes to 8%. Higher yields are great, but we start running into sustainability issuers the higher we go. The NAV rate comes to 7.72%.

Last year we saw a small special distribution from the fund of \$0.0621. This came as they realized many of the gains that they carried over from 2021. It wasn't a function of actually good performance in 2021; as we touched on above, they produced losses for the year. That strong 2020 performance was exactly why we saw a boost to the distribution in 2021 too.

BMEZ covers its distribution through capital gains. It earns no net investment income. NII is simply the total income minus the expenses. Since the fund is focused on innovation and private companies, it doesn't collect a lot of dividends or interest.

	BMEZ	
	Six Months Ended 06/30/21 (unaudited)	Period from 01/30/20 ^(a) to 12/31/20
INCREASE (DECREASE) IN NET ASSETS		
OPERATIONS		
Net investment loss	\$ (17,812,141)	\$ (24,489,080)
Net realized gain (loss)	181,095,794	131,179,423
Net change in unrealized appreciation (depreciation).....	<u>(110,009,621)</u>	<u>1,203,954,044</u>
Net increase (decrease) in net assets resulting from operations	<u>53,274,032</u>	<u>1,310,644,387</u>
DISTRIBUTIONS TO SHAREHOLDERS^(b)		
Decrease in net assets resulting from distributions to shareholders	<u>(87,882,464)</u>	<u>(101,402,843)</u>
CAPITAL SHARE TRANSACTIONS		
Net proceeds from the issuance of shares	—	2,253,296,500
Reinvestment of distributions	—	—
Net increase in net assets derived from capital share transactions.....	<u>—</u>	<u>2,253,296,500</u>
NET ASSETS		
Total increase (decrease) in net assets	(34,608,432)	3,462,538,044
Beginning of period	<u>3,462,638,044</u>	<u>100,000</u>
End of period	<u>\$ 3,428,029,612</u>	<u>\$ 3,462,638,044</u>

BMEZ Semi-Annual Report (BlackRock)

Now that the gains are drying up, we could see a distribution cut. All this being said, it will remain one of the only ways to continue to get paid until a rebound. The fund will ultimately continue to pay at least something due to the CEF structure.

BMEZ's Portfolio

The average market cap of the underlying investments in its portfolio comes to \$21.724 billion. The average market cap of the underlying holdings for BME comes to \$167 billion. That just shows that BMEZ is investing in a lot smaller companies relative to BME. Smaller companies tend to be riskier, which is the case with BMEZ. That's what makes it a riskier CEF in the first place. Then we have the fund's private holdings as well.

At the end of Q3 2021, most of their private investments have been oriented towards biotech stocks. No surprise, this is one of the worst areas of the market - that's why BMEZ has performed so poorly.

Fortunately for us, many of the private holdings BMEZ had invested in have gone public now. That means we can take a look at how some of them have fared.

Company	GICS Industry	Investment Quarter	Investment Amount (\$m)	Current Phase	Portfolio Ownership
Oak Street	Healthcare Providers & Services	Q1 2020	\$97.4	Public	Held
Kymera Therapeutics	Biotechnology	Q1 2020	\$23.5	Public	Held
Sigilon Therapeutics	Biotechnology	Q1 2020	\$3.0	Public	Held
Annexon	Biotechnology	Q2 2020	\$15.3	Public	Held
Taysha Gene Therapies	Biotechnology	Q3 2020	\$4.0	Public	Held
Everest Medicine	Pharmaceuticals	Q2 2020	\$21.1	Public	Held
Goldfinch	Biotechnology	Q2 2020	\$3.0	Private	Held
Insitro	Pharmaceuticals	Q2 2020	\$8.0	Private	Held
Antengene	Pharmaceuticals	Q3 2020	\$7.1	Public	Held
Kronos Bio, Inc.	Biotechnology	Q3 2020	\$4.6	Public	Held
Talaris Therapeutics	Biotechnology	Q3 2020	\$7.4	Public	Held
Olema	Biotechnology	Q3 2020	\$2.9	Public	Held
LianBio	Biotechnology	Q4 2020	\$6.0	Private	Held
Decibel Therapeutics	Biotechnology	Q4 2020	\$3.9	Public	Held
Ambrx, Inc.	Biotechnology	Q4 2020	\$14.6	Public	Held
Imago Biosciences, Inc.	Biotechnology	Q4 2020	\$10.0	Public	Held
Acumen Pharmaceuticals, Inc.	Biotechnology	Q4 2020	\$3.3	Public	Held
Everly Well, Inc.	Healthcare Providers & Services	Q4 2020	\$10.0	Private	Held
Connect Biopharmaceuticals	Biotechnology	Q4 2020	\$14.6	Public	Held
Neurogene	Biotechnology	Q4 2020	\$9.6	Private	Held
Immuneering	Biotechnology	Q4 2020	\$6.0	Public	Held
Immunocore	Biotechnology	Q4 2020	\$5.9	Public	Held
Abbisko	Biotechnology	Q4 2020	\$14.8	Private	Held
Adicon	Healthcare Providers & Services	Q4 2020	\$17.8	Private	Held
IsoPlexis Corp	Biotechnology	Q4 2020	\$14.8	Private	Held
Affinivax, Inc.	Biotechnology	Q1 2021	\$5.8	Private	Held
TScan Therapeutics	Biotechnology	Q1 2021	\$10.0	Public	Held
Cellarity, Inc.	Biotechnology	Q1 2021	\$14.6	Private	Held
Design Therapeutics	Biotechnology	Q1 2021	\$10.0	Public	Held
Vividion Therapeutics	Pharmaceuticals	Q1 2021	\$8.0	Private	Held
Amunix, Inc	Biotechnology	Q1 2021	\$10.0	Private	Held
OnKure, Inc.	Biotechnology	Q1 2021	\$2.3	Private	Held
Omega Therapeutics	Biotechnology	Q1 2021	\$8.0	Public	Held
Monte Rosa	Biotechnology	Q1 2021	\$8.0	Public	Held
Rapid Micro Biosystems	Biotechnology	Q1 2021	\$9.9	Public	Held
Nucleix Limited	Medical Devices & Supplies	Q1 2021	\$6.9	Private	Held
Numab Therapeutics	Biotechnology	Q2 2021	\$7.6	Private	Held
Bright Peak Therapeutics	Biotechnology	Q2 2021	\$8.0	Private	Held
Nikang Therapeutics	Biotechnology	Q2 2021	\$6.0	Private	Held
PSI Quantum	Semiconductors	Q2 2021	\$15.0	Private	Held
Quanta Dialysis Tech	Healthcare Providers & Services	Q2 2021	\$22.0	Private	Held
eXo Imaging, Inc	Medical Devices & Supplies	Q2 2021	\$13.2	Private	Held
Company A	Healthcare Providers & Services	Q3 2021	\$17.1	Private	Held
Company B	Biotechnology	Q3 2021	\$13.5	Private	Held
Company C	Medical Devices & Supplies	Q3 2021	\$6.4	Private	Held

BMEZ Private Holdings (BlackRock)

As of January 20th, 2022, "pharma, biotech and life sciences" is the largest sector allocation of the fund. Which just reiterates what we were seeing in its private holdings.

as of Jan 20, 2022

Type	Fund ▾	% of Market Value
	Fund	
Pharma, Biotech & Life Sciences	51.42	
Health Care Equipment & Services	42.48	
Cash and/or Derivatives	2.73	
Diversified Financials	1.98	
Software & Services	0.66	
Semiconductors & Semiconductor Equipment	0.56	
Tech Hardware & Equipment	0.16	
Banks	0.00	
Energy	0.00	
Insurance	0.00	

[Show More](#)

BMEZ Sector Allocations (BlackRock)

One of the fund's largest private holdings was Oak Street ([OSH](#)). This stock was a 2020 darling when it went public. The [IPO price was \\$21](#), and it quickly shot up after its IPO. A large decline that began in August 2021 has sent the shares back below this price at below \$18 now.



Ycharts

Let's look at Kymera Therapeutics ([KYMR](#)). This was another fairly significant investment for BMEZ in the biotech space. This stock went [public in August 2020](#) at \$20 per share. We once again saw it open sharply higher. Then towards the end of 2020, it started to go parabolic. Since then, the stock has held above its IPO price but has been cut in more than half from its peak. Losses in the share price accelerated in the last few weeks.



Ycharts

Suffice it to say, a lot of the other charts are similar. Sigilon Therapeutics ([SGTX](#)), Annexon ([ANNX](#)) and Taysha Gene Therapies ([TSHA](#)) all went public at various times. SGTX has to be one of the worst, though; it is now a penny stock. ANNX and TSHA are heading that way.



Ycharts

Many of these stocks seem as though it could be hard to recover these losses as well. While the private investments of BMEZ make it more unique and interesting, it also shows how erratic, volatile and risky these things are.

as of Jan 20, 2022

Name	Weight (%)	Name	Weight (%)
SEAGEN INC	2.28	MASIMO CORPORATION	1.80
ALCON AG	2.24	TELEFLEX INCORPORATED	1.73
VERTEX PHARMACEUTICALS INCORPORATED	2.10	GENMAB A/S	1.68
EDWARDS LIFESCIENCES CORP	1.84	DAIICHI SANKYO CO LTD	1.55
RESMED INC	1.82	INTUITIVE SURGICAL INC	1.52

BMEZ Top Ten (BlackRock)

Though to be fair, the private holdings that more recently went public in 2020 aren't the only stocks struggling. BMEZ's top ten include several names that have been around for years. Of the top five, only Alcon ([ALC](#)) doesn't have more than 10+ years of being publicly traded. Here is a look at the performance of BMEZ's top 5 positions.

Seeking Alpha^α

Jan 23 2022, 4:32PM EST. Powered by YCHARTS

Ycharts

It isn't surprising to see why BMEZ has been having a tough go with performance such as this. A lot of what the fund invests in is just not performing. Positions providing results for shareholders are relatively few in this fund. I believe some of the private holdings at this point are a lost cause; however, others here seem just to be providing an attractive entry point. That's what makes BMEZ more interesting too.

Conclusion

BMEZ has been having a tough time since its early successes. Those successes translated into higher distribution for shareholders. However, that distribution seems to be under pressure now, with assets deflating rapidly for the fund. Most of the fund's private and public holdings all have the same trajectory - that is sharply higher than a rapid decline. The declines picked up more momentum as we head into 2022. It has been rebounding strongly, and I'm not sure if this is a turnaround or we'll head lower. However, I do believe that if one can handle the higher risks here, it is a tempting offer being served up.

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This article was written by



Nick Ackerman

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Nick Ackerman is a former financial advisor using his experience to provide coverage on closed-end funds and exchange-traded funds. Nick has previously held Series 7 and Series 66 licenses and has been investing personally for over 14 years.

He contributes to the investing group [CEF/ETF Income Laboratory](#) along with leader Stanford Chemist, and Juan de la Hoz and Dividend Seeker. They help members benefit from income and arbitrage strategies in CEFs and ETFs by providing expert-level research. The service includes: managed portfolios targeting safe 8%+ yields, actionable income and arbitrage recommendations, in-depth analysis of CEFs and ETFs, and a friendly community of over a thousand members looking for the best income ideas. These are geared towards both active and passive investors. The vast majority of their holdings are also monthly-payers, which is great for faster compounding as well as smoothing income streams. [Learn more](#).

Analyst's Disclosure: I/we have a beneficial long position in the shares of BMEZ, BME either through stock ownership, options, or other derivatives. I wrote this article myself, and it expresses my own opinions. I am not receiving compensation for it (other than from Seeking Alpha). I have no business relationship with any company whose stock is mentioned in this article.

This article was originally published to members of the CEF/ETF Income Laboratory on January 23rd, 2022.

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Home > ETFs and Funds Analysis > Closed End Funds Analysis

BMEZ: Healthcare Exposure With A ~4% Yield And Growth Potential

Jan. 30, 2021 4:05 AM ET | **BlackRock Health Sciences Term Trust (BMEZ)** | ANNX, ASA, BME... |
50 Comments | 14 Likes



Nick Ackerman

Investing Group Leader

Summary

- BMEZ delivered solid results throughout 2020, helped by the healthcare sector overall.
- This fund doesn't just invest in your typical healthcare companies though and tilts towards smaller and private investments.
- These companies are ripe for significant growth, at the same time, BMEZ allows investors to capitalize on this while still providing an attractive dividend.
- I am Nick Ackerman, a former financial advisor with 15+ years experience. I contribute to the investing group [CEF/ETF Income Laboratory](#) where I share exclusive high-yield opportunities in the CEF/ETF space.

Written by Nick Ackerman, co-produced by Stanford Chemist

BlackRock Health Sciences Trust II (NYSE:[BMEZ](#)) helped deliver some solid results to shareholders throughout 2020. If performance were listed on CEFConnect, it would be the fourth-best performing CEF in 2020. I believe that given the current conditions, the fund could be due for a distribution boost, too. It can continue to be a top-performer - even if it isn't returning nearly 60% again - by actively managing its portfolio. The exposure it gains through investing in private investments can be a boon for the fund.

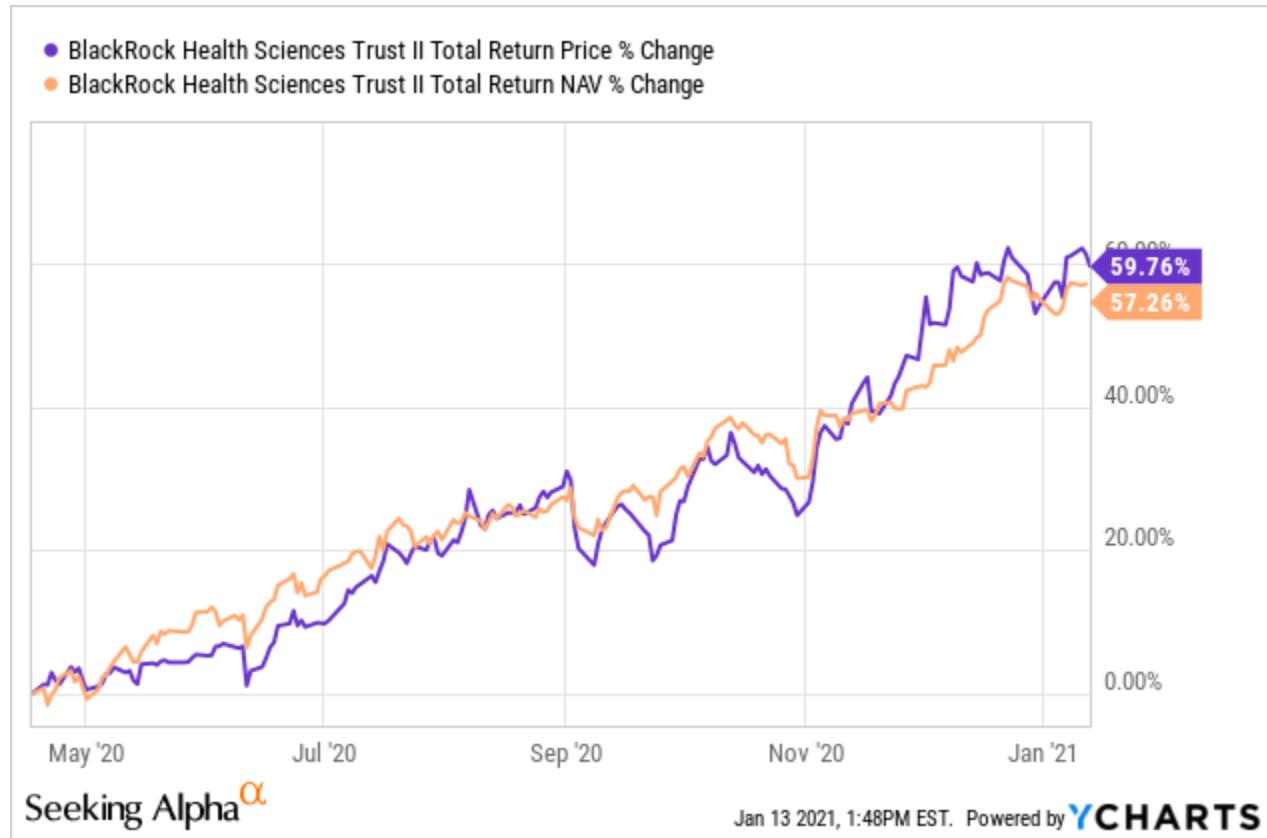
We first covered the fund on May 10, 2020, on Seeking Alpha. The returns since that time have been quite appealing. Naturally, as one would suspect with the fourth-best performing fund of 2020.

About This Article

Ticker Covered	BMEZ
Author's rating at publication	Neutral
Price at publication	\$19.95
Last price	\$29.77
Change since publication	49.20%
BMEZ total return since publication	54.44%
S&P 500 change since publication	30.82%
Days since publication	248

(Source - *Seeking Alpha*, as of January 13th, 2021)

That article was originally published to members of the CEF/ETF Income Laboratory on April 18th, 2020. Had one bought at that time, returns would have been even better. Though no surprise there as we were in full rebounding mode from the bear market.



Data by YCharts

BMEZ "seeks to invest up to 25% in private companies." It intends on doing this through "at least 80% of its total assets in equity securities of companies principally engaged in the health sciences group of industries and equity derivatives with exposure to the health sciences group of industries." With this, it also utilizes an options strategy. It last reported 21.84% of the portfolio being overwritten. This is below its target range of 30 to 40% and would indicate a bullish stance.

The fund is structured as a [limited-term fund](#), in that it will liquidate around January 29th, 2032. This is to ensure that investors can receive full NAV. This has become a popular way to launch funds as we typically see CEFs operating at perpetual discounts. Of course, that is one of the draws of CEFs is investing when they are at discounts. The term structure allows one to capitalize on the liquidation, however. Then an investor can potentially put funds back to work in another term fund or similar fund to still keep the exposure. With BMEZ, there isn't a fund that is too similar at this time.

Additionally, the fund may be changed to a perpetually trading vehicle after a tender offer of 100% of shares. Thus, one could reap the reward of the liquidation and then still have access to the fund should it continue to operate. The minimum is quite low to continue operation as well, as they put it at \$200 million. We should also see a declining discount as we reach nearer that date in 2032. I believe that unless performance turns terrible, that it will more than likely turn perpetual.

The fund is quite large at over \$3.5 billion in total managed assets. Though the performance since its launch has helped that significantly. The expense ratio is 1.30% - while that is higher than other BlackRock option-based funds, I suspect that it relates to the private investments. It would take considerably more amount of time researching and investing in private investments.

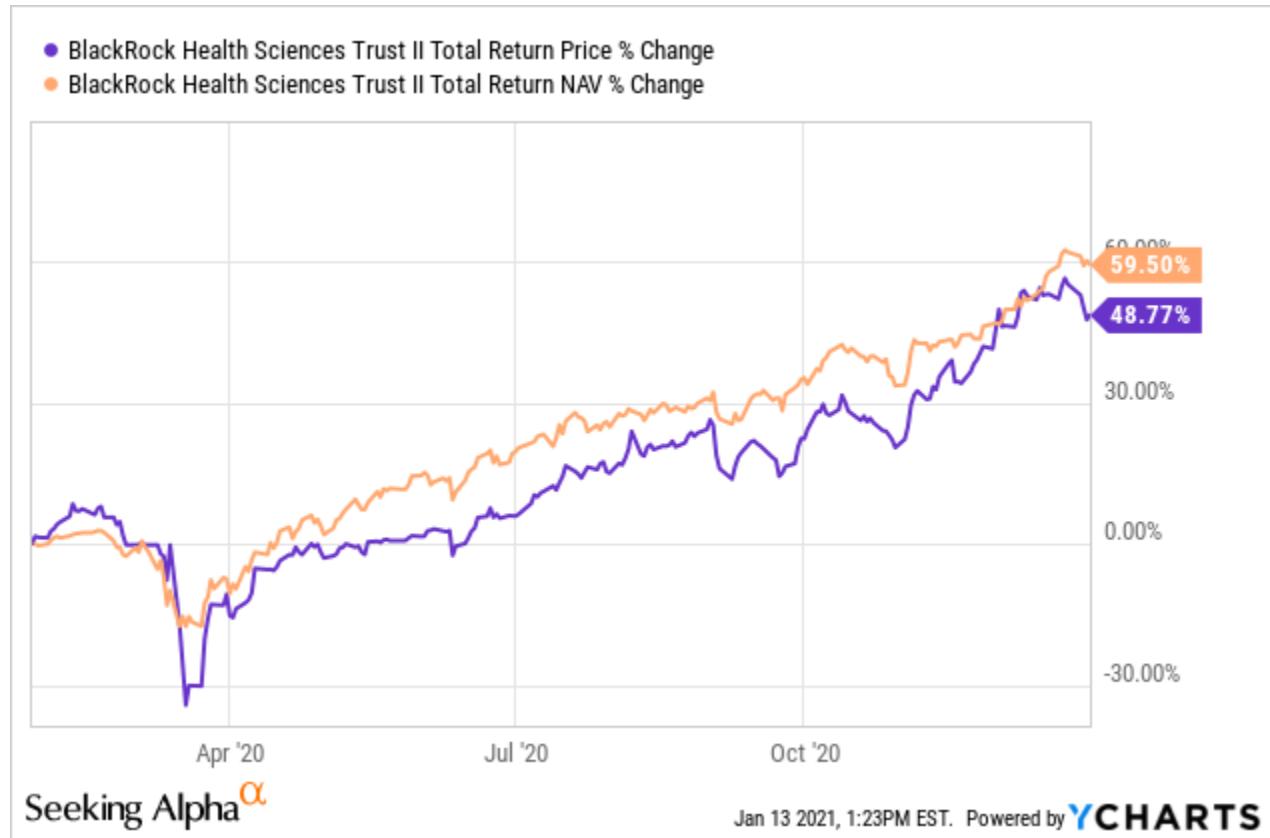
Performance - A Top Performer Of 2020, Bright Future Still Ahead

Beating BMEZ out for performance in 2020 on a total return NAV basis was the BlackRock tech funds; BlackRock Science and Technology Trust II ([BSTZ](#)) and BlackRock Science and Technology Trust ([BST](#)). Of course, those two had the massive benefit of investing in the tech space. Additionally, ASA Gold & Precious Metals ([ASA](#)) had also beaten the fund out in terms of performance. It just barely beat out ASA too. ASA put up 60.51% total NAV returns compared to 59.50% for BMEZ.

BMEZ was helped by its healthcare exposure. More specifically, it holds a portion of biotech. This was a [subsector of healthcare](#) that had done well throughout 2020 based on a huge push for vaccines. The focus on smaller companies that BMEZ does can also lead to the significant growth that turns into stock appreciation.

An additional factor to help boost the fund was that it launched on January 29th, 2020. This was merely a few weeks before the market started to tank - meaning it had a considerable amount of cash to put to work at the exact right time. This is why the fund's NAV hardly dropped at all during the panic of March selling.

Still, that healthcare wasn't the only reason that saw BMEZ outperform. After all, of the top 28 performers (BMEZ would have made it 29), there are no other healthcare names listed.



Data by YCharts

There were various times that one could buy BMEZ throughout the year at an attractive discount. That being said, the current 3.74% discount isn't anything to brush aside either. The other healthcare-related BlackRock Health Sciences ([BME](#)) predecessor to BMEZ trades at premiums regularly. In fact, its premium has been widening lately. Albeit they do invest differently - with BME in the more traditional healthcare names. BME also pays a slightly higher distribution rate currently. Based merely on the incredible returns of BMEZ.

The fund likely won't return nearly 60% in 2021 again. It doesn't need to. It can still be an attractive choice for investors looking for a bit of yield and growth potential. It can do so because it is continually looking for investment opportunities. It has the flexibility to invest where and when it wants. The fund's private investments skew towards biotech - this could potentially IPO and become huge winners. That is even despite whatever the broader market is doing. Though obviously, a stronger broader market will help it along. In that way, it is similar to BSTZ - where we might not see the typically higher yields in these funds - but also allowing for appreciation as well.

Distribution - Relatively Low, But Room To Grow

The fund launched with a distribution of \$0.10 per month. This has been maintained over the last year or so. This does work out to a relatively low 4.09% - which might not be too enticing for most CEF investors. This works out to 3.94% on a NAV basis. That being said, I believe there could be a good chance it boosts this in 2021. This would be based on if it starts realizing some of these underlying gains. When we last covered it, the fund had only realized losses on the books. However, it wasn't a significant amount compared to its unrealized gains.

BMEZ

Period from
01/30/20^(a)
to 06/30/20
(unaudited)

INCREASE (DECREASE) IN NET ASSETS

OPERATIONS

Net investment loss	\$ (7,119,605)
Net realized loss	(16,466,533)
Net change in unrealized appreciation (depreciation)	452,844,922
Net increase in net assets resulting from operations	<u>429,258,784</u>

DISTRIBUTIONS TO SHAREHOLDERS^(c)

From net investment income and net realized gain	(33,800,948) ^(d)
From return of capital	—
Decrease in net assets resulting from distributions to shareholders	<u>(33,800,948)</u>

CAPITAL SHARE TRANSACTIONS

Proceeds from shares sold	2,253,396,500
Redemption of shares resulting from share repurchase program (including transaction costs)	—
Net increase in net assets derived from capital share transactions	<u>2,253,396,500</u>

NET ASSETS

Total increase in net assets	2,648,854,336
Beginning of period	—
End of period	<u>\$2,648,854,336</u>

(Source - [Semi-Annual Report](#))

If it starts realizing some of these, to offset the realized losses, then to avoid being taxed, it will have to distribute them to shareholders. This could come in either the form of a higher monthly distribution rate or a year-end special to keep it in compliance. I would lean towards the fund increasing the monthly rate, though I'm biased in that I would prefer that. We also know that BlackRock isn't afraid to adjust distributions as needed. That is, both boosting or cutting.

Holdings - Potentially Greater Volatility For Potentially Greater Rewards

As mentioned previously, the fund does invest in smaller companies. Some of these are private investments leaning towards biotech holdings. This can cause greater volatility during times of panic. We hadn't really witnessed that during the last crash as the fund had a considerable amount of cash that it was still putting to work.

Private investments in BMEZ:³

Company	GICS Industry	Investment quarter	Investment amount (\$m)	Current phase	Portfolio ownership
Oak Street	Healthcare providers & services	Q1 2020	\$97.40	Public	Held
Kymera Therapeutics	Biotechnology	Q1 2020	\$23.50	Public	Held
Sigilon Therapeutics	Biotechnology	Q1 2020	\$3.00	Private	Held
Annexon	Biotechnology	Q2 2020	\$15.30	Public	Held
Taysha Gene Therapies	Biotechnology	Q3 2020	\$4.00	Public	Held
Everest Medicine	Pharmaceuticals	Q2 2020	\$21.10	Private	Held
Goldfinch	Biotechnology	Q2 2020	\$3.00	Private	Held
Insitro	Pharmaceuticals	Q2 2020	\$5.00	Private	Held
Company A	Pharmaceuticals	Q3 2020	\$7.10	Private	Held
Company B	Biotechnology	Q3 2020	\$4.60	Private	Held
Company C	Biotechnology	Q3 2020	\$7.40	Private	Held
Company D	Biotechnology	Q3 2020	\$2.90	Private	Held

Company names are not disclosed for positions initiated in the third quarter; these will be disclosed in the Q4 commentary.

(Source - [Q3 Commentary](#))

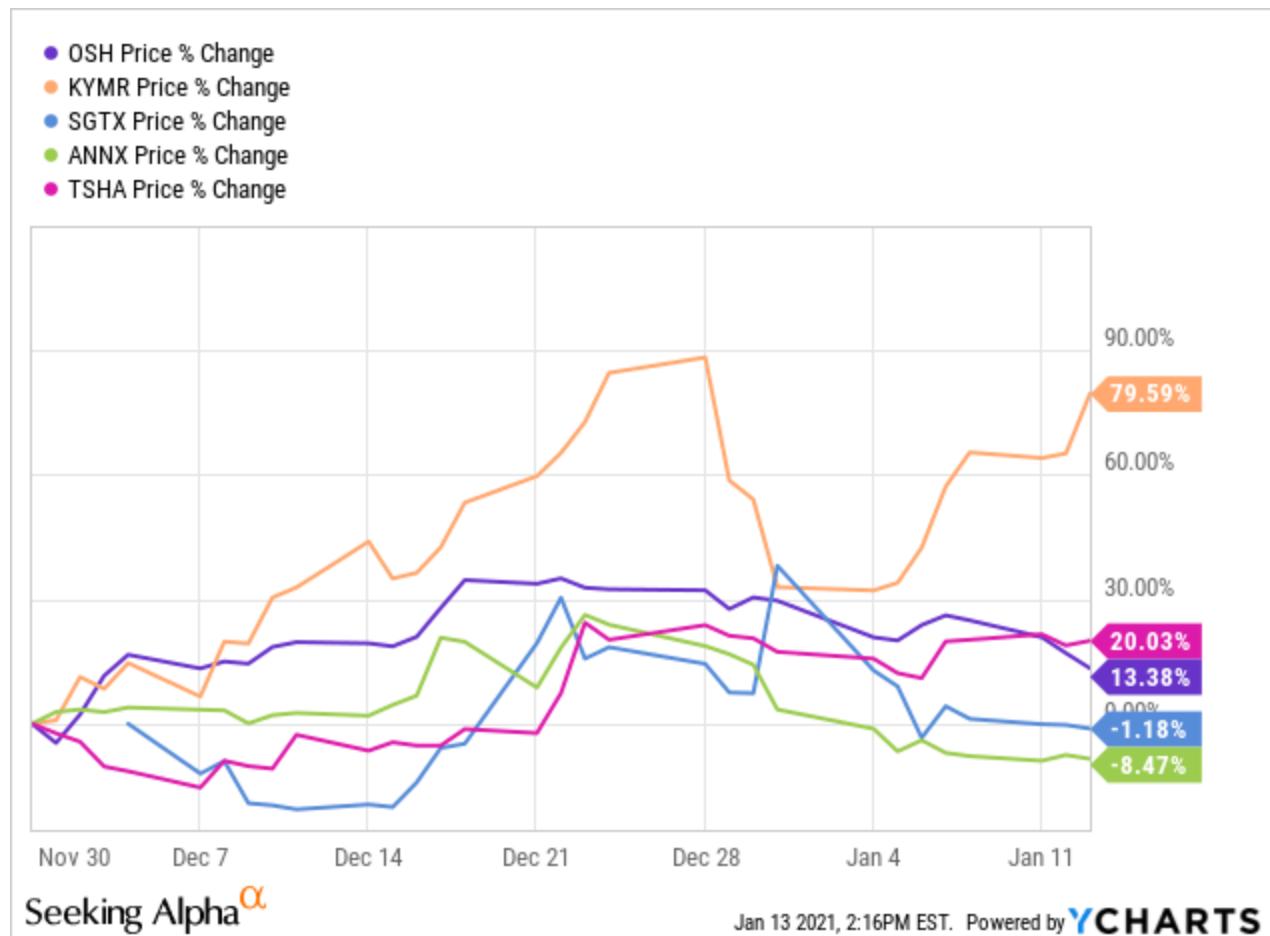
The latest commentary was for Q3. This was for the month end of September 2020. At that time, the fund listed that it had only 1.9% of total assets in private investments. That is a significant amount away from its target. This could be viewed as either positive or negative.

The positive would be that it is being pretty selective in what it chooses to put assets in. The negative could be that it is missing out on some opportunities by being too selective. I'm more inclined to be on the side of this being positive.

It noted that four of the fund's eight private investments had successful IPOs in the quarter.

Notably, four of the Trust's private investments underwent successful IPOs during the quarter, highlighting the strong market opportunity of pre-IPO companies. The Trust invested in an additional four private companies during the third quarter, all within the biotechnology industry, with a general focus on oncology and autoimmune diseases.

Since then, it would seem even more have gone the IPO route. This includes Oak Street Health, Inc ([OSH](#)), Kymera Therapeutics Inc ([KYMR](#)), Sigilon Therapeutics, Inc. ([SGTX](#)), Annexon Inc. ([ANNX](#)), Taysha Gene Therapies ([TSHA](#)) and Everest Medicines Ltd. (on the Hong Kong exchange). Basically, it is having a high success rate of IPO companies. To me, this makes sense as BlackRock has all the tools and insights behind the curtains of what is going on. I'm happy to tag along with the managers too.



Data by YCharts

It looks like since the initial launch, a couple of these have given up their gains (SGTX and ANNX). Still, not a bad outcome if we look at all the launches. KYMR being a huge outlier with an incredible performance of those listed.

Of these private investments, it would appear that OSH is the only one in the fund's top ten as of December 31st, 2020.

as of Dec 31, 2020

Name	Weight (%)	Name	Weight (%)
GENMAB A/S	3.36	TELEFLEX INCORPORATED	1.71
OAK STREET HEALTH INC.	3.30	PPD INC	1.68
SEAGEN INC	2.76	WUXI BIOLOGICS CAYMAN INC	1.64
AMEDISYS INC	1.95	LHC GROUP INC	1.52
INTUITIVE SURGICAL INC	1.76	ALCON AG	1.48

(Source - Fund Website)

Since we [last covered](#) the fund, there weren't significant changes. The top position remains Genmab ([GMAB](#)). Then we have OSH, which was the 3rd largest position now moved to the second largest. Seagen Inc. ([SGEN](#)) is the former Seattle Genetics but [changed its name](#) recently.

One of the only new names to appear is Wuxi Biologics Cayman Inc. ([OTCPK:WXXWY](#)). This replaced Masimo Corporation ([MASI](#)) in the top holdings list. WXXWY trades OTC. It is a Chinese company that "[offers](#) comprehensive, integrated and highly customizable services. The company offers multinational pharmaceutical and biotechnological companies in the world end-to-end solutions empowering anyone and any company to discover, develop and manufacture biologics from concept to commercial manufacturing."

China exposure is at 11.50%. Though I view this as a positive as it helps highlight that it isn't just interested in investing in U.S. companies. Reflecting that it will seek opportunities wherever they might be present. This greater flexibility could provide for more attractive returns. I'm inclined to believe that BlackRock has the tools and technology to invest with valuable insights not otherwise available to most.

as of Dec 31, 2020

Type	▲	Fund ►
United States		73.09
China		11.50
Denmark		5.93
Switzerland		4.17
Belgium		2.19
Germany		1.48
Cash and/or Derivatives		-1.29
Other		2.95

(Source - Fund Website)

That being said, it does still provide plenty of exposure to U.S. investments at this time. Though there is no **specific restriction** in investing globally in any allocation. With that, any time it updates its holdings, it could be a surprise. So far, it has stuck with mostly a heavier U.S. allocation.

The Trust may invest in companies of any market capitalization located anywhere in the world, including companies located in emerging markets. The Trust will focus its investments in mid- and small-capitalization companies. Foreign securities in which the Trust may invest may be U.S. dollar-denominated or non-U.S. dollar-denominated.

This is similar to BSTZ's strategy of not being artificially limited. Instead, allowed to invest where those managers see fit as well.

Conclusion

BMEZ is a solid choice for investors wanting some diversification in the healthcare space. The small to mid size and private investments will potentially make this fund more volatile. That is relatively speaking to more traditional healthcare exposure. Traditional healthcare exposure would be heavier in pharmaceutical companies that typically offer more stabilized earnings.

With BMEZ, an investor is getting a chance to invest in areas they otherwise wouldn't be with BlackRock's team running the ship. That to me is a big positive of this fund. I believe that almost any discount on this fund is attractive for a longer-term investor. The downside here is that with BMEZ, one is getting a lower yield. This does lead to potentially greater growth though. Which should then itself translate into the growth of its distribution too, when appropriate. Even a 4% yield is higher than what you would get with the underlying holdings, of which almost none pay dividends at all.

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CEF & ETF Income

This article was written by



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Nick Ackerman is a former financial advisor using his experience to provide coverage on closed-end funds and exchange-traded funds. Nick has previously held Series 7 and Series 66 licenses and has been investing personally for over 14 years.

He contributes to the investing group [CEF/ETF Income Laboratory](#) along with leader Stanford Chemist, and Juan de la Hoz and Dividend Seeker. They help members benefit from income and arbitrage strategies in CEFs and ETFs by providing expert-level research. The service includes: managed portfolios targeting safe 8%+ yields, actionable income and arbitrage recommendations, in-depth analysis of CEFs and ETFs, and a friendly community of over a thousand members looking for the best income ideas. These are geared towards both

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Analyst's Disclosure: I am/we are long BMEZ, BME, BSTZ, BST, ASA. I wrote this article myself, and it expresses my own opinions. I am not receiving compensation for it (other than from Seeking Alpha). I have no business relationship with any company whose stock is mentioned in this article.

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Insider Weekends: Cluster Of Insider Purchases At NextEra And Charter

Feb. 07, 2022 11:55 AM ET | **Charter Communications, Inc. (CHTR) Stock, NEE Stock** | ARES, ASAN, GOOG ... | 6 Comments | 13 Likes

**Asif Suria**[Investing Group Leader](#)

Summary

- A cluster of insider purchases at the utility NextEra Energy and the cable company Charter Communications.
- Four different insiders including both the CEO and CFO of NextEra Energy acquired shares after a rare large drop in the stock that saw it dip below the \$70 level.
- Charter Communications saw its first insider purchase in over four years.
- I am Asif Suria, a tech industry veteran and entrepreneur. I have 20 years of experience actively investing and I am the leader of the special situations focused investing group [Inside Arbitrage](#)



Ijubaphoto/E+ via Getty Images

Market volatility remained elevated last week with big swings in both directions and the Nasdaq dragged down by single-day drops of 25% or more in stocks like PayPal ([PYPL](#)) and Meta Platforms ([FB](#)). It certainly did not feel like the S&P 500 closed up for the week given the carnage underneath the surface, especially in tech stocks.

Expectations of a challenging market were one of the reasons we wrote about a company that acts as a market maker and benefits from volatility in December and the company is up more than 12% in our model portfolio despite the general market decline.

In my article titled "[Positions For 2022: A Changing Of The Guard](#)" at the start of the year, I wrote the following,

I think there are opportunities in certain sectors and heightened risks in others. I'm about 75% invested after scaling back on several positions and would be more comfortable at the 60% to 65% level at this stage of the market. The rest of the portfolio does not have to be in low yielding cash but can instead be in relatively safe merger arbitrage positions to generate yield and to provide ballast during rough seas.

I have been very tempted to start buying after this recent pullback but have been showing restraint. I did write cash secured put options on Doximity ([DOCS](#)) and have been considering starting a new position in AirBnB ([ABNB](#)) or adding to my position in Twitter ([TWTR](#)), which took another hit after the disappointment results from Meta Platforms. We wrote the following two weeks ago in our Insider Weekends post [discussing the RV manufacturer Thor Industries](#),

What we saw in the markets this week was particularly worrisome because the sell off was broad-based and even asset classes with low correlations between them appeared to be down. We are currently seeing mean reversion in full swing. As market participants who have invested through multiple cycles know, mean reversion often overshoots the mean. In other words, stocks can go from "strong buy" to "screaming buy" to "how can it get any cheaper" before they bottom. We haven't yet approached the screaming buy phase yet as folks are already lining up to buy the dip, which has worked very well during the last decade.

I don't think we see the quick rebound this time like we did after the COVID-19 related drop in early 2020. With monetary stimulus and quantitative easing out of the picture, all we have left now is fiscal stimulus in the form of the Build Back Better infrastructure plan, which still needs to win approval in the Senate.

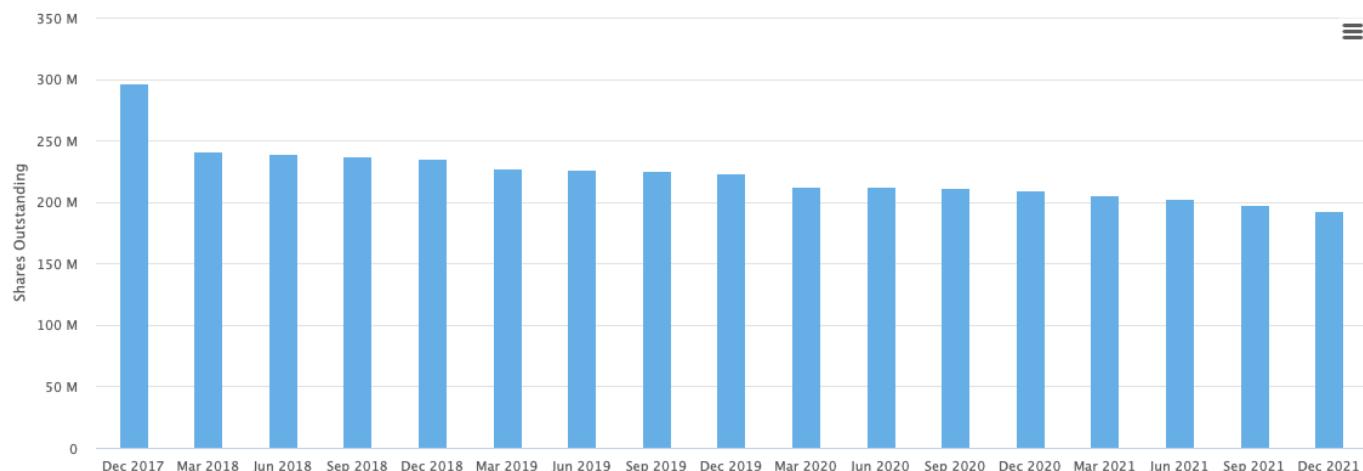
Last week we saw an eclectic mix of insider purchases with a cluster of insider purchases at the utility NextEra Energy (NYSE:[NEE](#)) and the cable company Charter Communications (NASDAQ:[CHTR](#)) seeing its first insider purchase in over four years. Four different insiders including both the CEO and CFO of NextEra Energy acquired shares after a rare large drop in the stock that saw it dip below the \$70 level. The drop in the stock was a result of Q4 2021 revenue of \$5.05 billion coming in well below expectations of \$5.79 billion and the [sudden departure](#) of its Chairman and CEO [Jim Robo](#).

Mr. Robo has been with NextEra for 20 years and has served as its CEO since July 2012. He has been credited with transforming a sleepy electric utility into the clean energy juggernaut it is perceived as now. The company has been growing its dividend for 26 consecutive years and has an impressive 5 year dividend CAGR of 12%. The current yield is 2% with a 61% payout ratio. The company has been afforded a valuation premium compared to its peers and currently trades at a forward P/E of 27 and a forward EV/EBITDA of 16.49, not exactly the kind of valuation you would expect from a regulated utility in a rising interest rate environment. With \$54 billion in net debt on the balance sheet and management changes on the horizon, I would approach NextEra with caution despite insider enthusiasm.

Another heavily indebted company that saw insider buying was Charter Communications, which saw its CEO purchase \$1.63 million worth of stock. While we saw several insiders exercise options without selling them, which we view as a positive signal, this is the first open market insider purchase since October 2017. For a company with a market cap of \$124 billion, Charter has an astounding \$91 billion in net debt on its balance sheet. This accumulation of debt appears to be part of an intentional capital allocation strategy during a low interest rate environment with the company acquiring its larger rival Time Warner Cable in 2015 for \$78.7 billion and the acquisition of Bright House Networks for \$10.4 billion. The company has also been a notable cannibal having purchased over a third of its shares outstanding in the last four years as you can see below.

Charter Communications has been growing its revenue in the mid to high single digits, but generating big gains in earnings year-over-year. The company currently trades at a forward EV/EBITDA of 9.25 and a forward P/E of 20. In a bandwidth hungry world, Charter could remain a steady performer unless disrupted by something like [SpaceX's Starlink](#) satellite internet system that is now available to consumers.

CHANGE IN SHARES OUTSTANDING



Charter Communications Change in Shares Outstanding (InsideArbitrage.com)

(source: InsideArbitrage.com database)

Welcome to edition 605 of Insider Weekends. Insider buying increased last week with insiders purchasing \$176.12 million of stock compared to \$140.34 million in the week prior. Selling also increased to \$917.47 million compared to \$301.25 million in the week prior.

Sell/Buy Ratio:

The insider Sell/Buy ratio is calculated by dividing the total insider sales in a given week by total insider purchases that week. The adjusted ratio for last week went up to 5.21. In other words, insiders sold more than 5 times as much stock as they purchased. The Sell/Buy ratio this week was unfavorable compared to the prior week when the ratio stood at 2.15.

Notable Insider Buys:

1. Asana, Inc. ([ASAN](#)): \$48.11

President, CEO, & Chair Dustin A. Moskovitz acquired 1,250,000 shares of this work management platform, paying \$48.15 per share for a total amount of \$60.19 million. Mr. Moskovitz increased his stake by 8.15% to 16,589,676 shares with this purchase.

P/E: N/A	Forward P/E: -49.09	Industry P/E: 56.34
P/S: 26.78	Price/Book: 36.2	EV/EBITDA: -38.07
Market Cap: \$8.97B	Avg. Daily Volume: 4,112,880	52 Week Range: \$25.41 – \$145.79

2. NextEra Energy, Inc.: \$76.01

Shares of this regulated electric company were acquired by 4 insiders:

- Chairman, President & CEO James L. Robo acquired 64,691 shares, paying \$77.26 per share for a total amount of \$4.99 million. Mr. Robo increased his stake by 5.65% to 1,209,774 shares with this purchase.
- EVP, Finance & CFO Rebecca J. Kujawa acquired 7,000 shares, paying \$71.83 per share for a total amount of \$502,810. Ms. Kujawa increased her stake by 15.71% to 51,547 shares with this purchase.
- Director Sherry S. Barrat acquired 2,000 shares, paying \$71.57 per share for a total amount of \$143,150. Mr. Barrat increased his stake by 1.82% to 111,674 shares with this purchase.
- Director Amy B. Lane acquired 700 shares, paying \$71.64 per share for a total amount of \$50,148. Ms. Lane increased her stake by 1.90% to 37,555 shares with this purchase.

P/E: 41.99	Forward P/E: 25.17	Industry P/E: 22.11
P/S: 8.74	Price/Book: 4.09	EV/EBITDA: 29.22
Market Cap: \$149.14B	Avg. Daily Volume: 9,221,832	52 Week Range: \$68.33 – \$93.73

3. Taysha Gene Therapies, Inc. ([TSHA](#)): \$7.94

Shares of this biotech company were acquired by 3 insiders:

- Director Paul B. Manning acquired 201,602 shares, paying \$7.80 per share for a total amount of \$1.57 million. Mr. Manning increased his stake by 10.67% to

2,091,704 shares with this purchase. These shares were purchased indirectly by BKB Growth Investments, LLC.

- Director Sukumar Nagendran acquired 10,000 shares, paying \$8.01 per share for a total amount of \$80,100. Mr. Nagendran increased his stake by 52.01% to 29,226 shares with this purchase.
- Director Phillip B. Donenberg acquired 3,000 shares, paying \$7.78 per share for a total amount of \$23,340.

P/E: N/A

Forward P/E: -1.58

Industry P/E: 142.83

P/S: N/A

Price/Book: 2.18

EV/EBITDA: -1.21

Market Cap: \$305.48M

Avg. Daily Volume: 196,308

52 Week Range: \$6.57 – \$31.6

4. Charter Communications, Inc.: \$608.92

Chief Operating Officer Christopher L. Winfrey acquired 2,750 shares of this cable services provider, paying \$591.96 per share for a total amount of \$1.63 million. Mr. Winfrey increased his stake by 5.91% to 49,256 shares with this purchase.

P/E: 24.88

Forward P/E: 15.94

Industry P/E: 17.10

P/S: 2.04

Price/Book: 7.49

EV/EBITDA: 9.85

Market Cap: \$105.19B

Avg. Daily Volume: 1,255,253

52 Week Range: \$549.59 – \$825.62

5. Western Alliance Bancorporation ([WAL](#)): \$97.91

Shares of this regional bank were acquired by 2 insiders:

- President and CEO Kenneth Vecchione acquired 5,600 shares, paying \$97.88 per share for a total amount of \$548,112. Mr. Vecchione increased his stake by 1.75% to 326,387 shares with this purchase.
- Vice Chairman and CFO Dale Gibbons acquired 5,000 shares, paying \$99.68 per share for a total amount of \$498,400. Mr. Gibbons increased his stake by 2.15% to

237,332 shares with this purchase.

P/E: 11.82	Forward P/E: 8.68	Industry P/E: 10.86
P/S: 5.78	Price/Book: 2.4	EV/EBITDA: N/A
Market Cap: \$10.44B	Avg. Daily Volume: 835,524	52 Week Range: \$73.89 – \$124.93

You can view the full list of purchases from this [Insider Buying](#) page.

Notable Insider Sales:

1. Alphabet Inc. ([GOOG](#)): \$2860.32

Shares of Alphabet were sold by 3 insiders:

- Director Lawrence Page sold 27,778 shares for \$2923.44, generating \$81.21 million from the sale.
- Chief Executive Officer Sundar Pichai sold 3,000 shares for \$2992.62, generating \$8.98 million from the sale.
- VP, Chief Accounting Officer Amie Thuener O'toole sold 42 shares for \$2755.81, generating \$115,744 from the sale.

P/E: 25.49	Forward P/E: 21.25	Industry P/E: 24.60
P/S: 7.35	Price/Book: 7.53	EV/EBITDA: 19.52
Market Cap: \$1.89T	Avg. Daily Volume: 1,345,480	52 Week Range: \$2002.02 – \$3042

2. Ares Management Corporation ([ARES](#)): \$79.39

Co-Founder, CEO and President Michael J. Arougheti sold 440,226 shares of this asset management company for \$79.02, generating \$34.79 million from the sale. 101,394 of these shares were sold indirectly by Atticus Enterprises LLC.

P/E: 42.3	Forward P/E: 24.5	Industry P/E: 14.77
P/S: 3.82	Price/Book: 7.75	EV/EBITDA: 31.99
Market Cap: \$13.58B	Avg. Daily Volume: 684,864	52 Week Range: \$47.25 – \$90.08

3. The Charles Schwab Corporation ([SCHW](#)): \$91.9

Shares of Charles Schwab were sold by 3 insiders:

- Chairman Charles R. Schwab sold 239,980 shares for \$89.97, generating \$21.59 million from the sale. These shares were sold indirectly by a trust.
- MD, EVP, and CFO Peter B. Crawford sold 6,000 shares for \$89.38, generating \$536,264 from the sale. *These shares were sold as a result of exercising options immediately prior to the sale.*
- MD, EVP, and General Counsel Peter J. Morgan III sold 2,239 shares for \$87.14, generating \$195,104 from the sale.

P/E: 32.47	Forward P/E: 18.95	Industry P/E: 14.77
P/S: 9.38	Price/Book: 3.66	EV/EBITDA: N/A
Market Cap: \$173.75B	Avg. Daily Volume: 6,887,217	52 Week Range: \$54.98 – \$95.62

4. Horizon Therapeutics Public Limited Company ([HZNP](#)): \$92.55

Chairman, President and CEO Timothy P. Walbert sold 125,000 shares of this biotech company for \$91.48, generating \$11.43 million from the sale. 25,000 of these shares were sold as a result of exercising options immediately prior to the sale.

P/E: 39.32	Forward P/E: 15.77	Industry P/E: 142.83
P/S: 7.1	Price/Book: 4.71	EV/EBITDA: 36.27
Market Cap: \$20.99B	Avg. Daily Volume: 1,493,130	52 Week Range: \$79.81 – \$120.54

5. Upstart Holdings, Inc. ([UPST](#)): \$98.63

Shares of this cloud-based artificial intelligence (AI) lending platform were sold by 2 insiders:

- Chief Executive Officer Dave Girouard sold 91,665 shares for \$109.37, generating \$10.03 million from the sale. 8,332 of these shares were sold indirectly by various trusts.
- General Counsel Alison Nicoll sold 7,500 shares for \$108.26, generating \$811,972 from the sale.

P/E: 99.73	Forward P/E: 42.33	Industry P/E: 8.29
P/S: 12.74	Price/Book: 11.18	EV/EBITDA: 77.61
Market Cap: \$8.08B	Avg. Daily Volume: 6,073,880	52 Week Range: \$42.511 – \$401.49

You can view the full list of sales from this [Insider Sales](#) page.

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Asif Suria is an entrepreneur and investor with a professional background in technology and a focus on event driven strategies including: merger arbitrage, spinoffs, (legal) insider trading, buybacks and SPACs. Asif has been actively investing for over 20 years and sharing his ideas for the past 10.

He is the leader of the investing group [Inside Arbitrage](#) where he shares investment ideas rarely found in mainstream financial press. Inside Arbitrage provides access to six different event-driven strategies to expand your investing toolbox, special situations focused tools, qualitative writeups of ideas through weekly articles, and a comprehensive monthly newsletter. [Learn more.](#)

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U.S. IPO Week Ahead: The September IPO Calendar Stays Good(Rx) And Busy In An 8-IPO Week

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Summary

- In another busy week for the September IPO market, eight IPOs are scheduled to raise \$2.0 billion, led by prescription drug marketplace GoodRx Holdings.
- Brazilian energy solutions provider Hygo Energy Transition plans to raise \$450 million at a \$2.4 billion market cap.
- Street research is expected for two companies in the week ahead.

In another busy week for the September IPO market, eight IPOs are scheduled to raise \$2.0 billion, led by prescription drug marketplace GoodRx Holdings ([GDRX](#)).

The largest deal of the week, GoodRx plans to raise \$900 million at an \$11.2 billion market cap. The company operates in the large prescription drug market, providing a mobile app and website that allows consumers to easily compare drug prices, and has delivered strong growth over the last several years. While lower-margin offerings have weighed on gross margin, GoodRx is profitable on an EBITDA and net basis and has generated strong cash flow.

Brazilian energy solutions provider Hygo Energy Transition ([HYGO](#)) plans to raise \$450 million at a \$2.4 billion market cap. Hygo Energy Transition provides integrated downstream liquefied natural gas solutions and has historically derived the majority of its revenue from LNG carriers, which it expects to convert into floating storage and regasification units. The company achieved profitability on an EBITDA basis in the 1H20, though revenue has been declining.

Bentley Systems ([BSY](#)), which provides software for construction and infrastructure projects, plans to raise \$194 million at a \$4.9 billion market cap. The IPO float is just 4.1% of the basic shares outstanding, or 3.3% after indicated buying. Operating in a cyclical market, the company has delivered profitability with strong margins. The company's operations were impacted by COVID-19 as usage declined due to delays in construction projects. Bentley Systems plans to pay a quarterly dividend.

Computer and gaming hardware supplier Corsair Gaming ([CRSR](#)) plans to raise \$238 million at a \$1.7 billion market cap. Corsair is a leading provider of high-performance gear for gamers and content creators, with a growing share in the \$30+ billion global market. The company has a track record of positive EBITDA, and gross margin has improved due to a higher mix of peripherals sales, though it faces exposure to volatile raw material costs.

Preclinical biotech Taysha Gene Therapies ([TSHA](#)) plans to raise \$125 million at a market cap of \$725 million. The company is developing gene therapies for a variety of rare genetic diseases. It plans to initiate a Phase 1/2 trial for its lead candidate under a CTA in Canada by the end of 2020. In addition, it plans to submit INDs for four additional programs to the FDA by the end of 2021.

Plant-based beverage manufacturer Laird Superfood ([LSF](#)) plans to raise \$42 million at a \$167 million market cap. Founded by surfers Laird Hamilton and Paul Hodge, Laird Superfood manufactures and markets differentiated plant-based and functional foods, generating a majority of revenue from its Superfood Creamer coffee creamers. The company has demonstrated strong growth but is unprofitable, and gross margin declined in the 1H20.

Amesite ([AMST](#)), which provides an AI-driven platform for developing online learning products, plans to raise \$16 million at a \$109 million market cap. Amesite provides customized, high performance, and scalable online products for customers, including businesses, universities and colleges, and K-12 schools. The company has a short operating history in online programs and generated less than \$60,000 in revenue in the FY20.

Phase 2 biotech PaxMedica ([PXMD](#)) plans to raise \$15 million at a market cap of \$59 million. The company's lead candidate PAX-101, an intravenous formulation of suramin, is currently in a Phase 2B clinical trial at six sites in South Africa for the treatment of autism spectrum disorder (ADS), with data expected in early 2021.

U.S. IPO Calendar

Issuer Business	Deal Size Market Cap	Price Range Shares Filed	Top Bookrunners
Amesite Detroit, MI	\$16M \$109M	\$4.50 - \$5.50 3,100,000	Laidlaw (UK)
Provides an AI-driven platform for developing online learning products.			
Bentley Systems Exton, PA	\$194M \$4,916M	\$17 - \$19 10,750,000	Goldman BofA
Provides software for construction and infrastructure projects.			
Corsair Gaming Fremont, CA	\$238M \$1,680M	\$16 - \$18 14,000,000	Goldman Barclays
Designs and supplies personal computer and gaming hardware components.			
GoodRx Santa Monica, CA	\$900M \$11,160M	\$24 - \$28 34,615,384	Morgan Stanley Goldman
Operates a prescription drug price comparison platform.			
Laird Superfood Sisters, OR	\$42M \$167M	\$18 - \$20 2,200,000	Canaccord Craig-Hallum
Manufactures plant-based packaged beverage products.			
Taysha Gene Therapies Dallas, TX	\$125M \$725M	\$18 - \$20 6,578,950	Goldman Morgan Stanley
Preclinical biotech developing gene therapies for very rare CNS disorders.			
Hygo Energy Transition Hamilton, Bermuda	\$450M \$2,400M	\$18 - \$21 23,100,000	Morgan Stanley Goldman
Provides integrated downstream liquefied natural gas solutions in Brazil.			
PaxMedica Woodcliff Lake, NJ	\$15M \$59M	\$5.50 - \$6.50 2,500,000	Benchmark
Phase 2 biotech developing therapies for neurodevelopmental disorders.			

Street research is expected for two companies in the week ahead. On Monday, 9/21: XPeng ([XPEV](#)). On Wednesday, 9/23: GreenPower Motor ([GP](#)).

IPO Market Snapshot

The Renaissance IPO Indices are market cap weighted baskets of newly public companies. As of 9/17/20, the Renaissance IPO Index was up 59.2% year-to-date, while the S&P 500 was up 3.9%. Renaissance Capital's IPO ETF (NYSE: IPO) tracks the index, and top ETF holdings include Zoom Video ([ZM](#)) and Uber ([UBER](#)). The Renaissance International IPO Index was up 35.5% year-to-date, while the ACWX was down 2.8%. Renaissance Capital's International IPO ETF (NYSE: IPOS) tracks the index, and top ETF holdings include Xiaomi and Meituan-Dianping.

[Original Post](#)

Editor's Note: The summary bullets for this article were chosen by Seeking Alpha editors.

This article was written by



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U.S. IPO Weekly Recap: Health And Tech Dominate The Calendar In An 11 IPO Week

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Summary

- September activity remained high as 11 IPOs and eight SPACs went public this past week.
- The IPO market shows no signs of slowing with eight IPOs and 18 SPACS submitting initial filings.
- Eight SPACs raised \$2.0 billion this past week.

September activity remained high as 11 IPOs and eight SPACs went public this past week. The IPO market shows no signs of slowing with eight IPOs and 18 SPACS submitting initial filings.

Prescription drug price comparison platform GoodRx ([GDRX](#)) priced well above the range to raise \$1.1 billion at a \$14.2 billion market cap. The company provides one of the leading prescription discount platforms with 4.4 million monthly active customers. GoodRx offers a combination of solid growth, profitability, and strong cash flow generation. GoodRx finished up 11%.

Gaming hardware maker Corsair Gaming ([CRSR](#)) priced at the midpoint to raise \$238 million at a \$1.7 billion market cap. Corsair Gaming is the leading provider of high-performance gear for gamers and content creators. Benefiting from the pandemic's social distancing, Corsair reached top-line growth of 58% and an EBITDA margin of 13% in the 2Q20. Corsair Gaming finished up 11%.

Construction software provider Bentley Systems ([BSY](#)) priced above the upwardly revised range to raise \$237 million at a \$6.7 billion market cap. With strong margins and cash flow, the company provides software used in the design, construction, and operation of infrastructure. Bentley Systems finished up 9%.

Precision oncology biotech PMV Pharmaceuticals ([PMVP](#)) priced at the high end to raise \$212 million at an \$836 million market cap. The company is early stage and has yet to begin clinical trials. PMV is initially pursuing a tumor-agnostic development strategy and submitted an IND for its lead candidate in August 2020, with a Phase 1/2 trial planned for the 2H20. PMV Pharmaceuticals finished up 108%.

Phase 1 biotech Prelude Therapeutics ([PRLD](#)) priced at the high end to raise \$158 million at a \$886 million market cap. The company's first clinical candidate is currently in a Phase 1 trial in select solid tumors and myeloid malignancies, and the company has observed early signs of clinical activity. It expects clinical data in the 1H21. Prelude Therapeutics finished up 38%.

Preclinical biotech Taysha Gene Therapies ([TSHA](#)) upsized and priced at the high end to raise \$157 million at a \$789 million market cap. This biotech is developing gene therapies to address a variety of rare, life-threatening genetic diseases. The company plans to initiate a Phase 1/2 trial for its lead candidate under a CTA in Canada by the end of 2020. Taysha Gene Therapies finished up 2%.

German display solutions provider VIA optronics ([VIAO](#)) priced at the low end to raise \$94 million at a \$338 million market cap. The company provides a wide range of customized display solutions, including curved display panels and solutions integrating multiple display touch assemblies under a single cover lens. Unprofitable, the company saw revenue fall in 2019 and the 1H20. VIA optronics finished down 32%.

Ocular disease biotech Graybug Vision ([GRAY](#)) upsized and priced at the midpoint to raise \$90 million at a \$353 million market cap. The company's lead candidate is an intravitreal injection being developed as a once-every-six months treatment for wet age-related macular degeneration (wet AMD) and diabetic macular edema (DME). The candidate is currently in a Phase 2b trial for wet AMD, with topline data expected in the 1H21. Graybug Vision finished up 3%.

Plant-based beverage producer Laird Superfood ([LSF](#)) priced at the high end of the upwardly revised range to raise \$58 million at a \$192 million market cap. Founded by surfers Laird Hamilton and Paul Hodge, Laird Superfood manufactures and markets differentiated plant-based and functional foods. The company has demonstrated strong growth but is unprofitable, and gross margin declined in the 1H20. Laird Superfood finished up 20%.

Amesite ([AMST](#)), which provides an AI-driven platform for developing online learning products, priced at the midpoint to raise \$15 million at a \$109 million market cap. The company is fast-growing but generated less than \$60,000 in revenue in the FY20, and it has a short operating history in online programs. Amesite finished up 4%.

Phase 3 biotech Greenwich LifeSciences ([GLSI](#)) priced at the midpoint to raise \$7 million at a \$78 million market cap. The company is developing GP2, an immunotherapy designed to prevent the recurrence of breast cancer following surgery, and it is planning to commence a Phase 3 trial in 2020. Greenwich LifeSciences finished down 13%.

Eight SPACs raised \$2.0 billion this past week. The pack was led by media-focused SPAC Falcon Capital Acq. (FCACU), Vector Capital's SPAC Vector Acquisition (VACQU), and Carnelian Energy Capital's SPAC Peridot Acquisition (PDACU), each of which raised \$300 million.

19 IPOs During the Week of September 21st, 2020

Issuer Business	Deal Size	Market Cap at IPO	Price vs. Midpoint	First Day Return	Return at 09/25
PMV Pharmaceuticals (PMVP)	\$212M	\$836M	6%	+108%	+108%
<i>Early stage biotech developing targeted therapies for cancer.</i>					
Laird Superfood (LSF)	\$58M	\$192M	16%	+85%	+105%
<i>Manufactures plant-based packaged beverage products.</i>					
Bentley Systems (BSY)	\$237M	\$6,700M	22%	+52%	+58%
<i>Provides software for construction and infrastructure projects.</i>					
GoodRx (GDRX)	\$1,142M	\$14,169M	27%	+53%	+58%
<i>Operates a prescription drug price comparison platform.</i>					
Prelude Therapeutics (PRLD)	\$158M	\$886M	6%	+38%	+38%
<i>Phase 1 biotech developing PRMT5 inhibitors for difficult to treat cancers.</i>					
Taysha Gene Therapies (TSHA)	\$157M	\$789M	5%	+20%	+22%
<i>Preclinical biotech developing gene therapies for very rare CNS disorders.</i>					
Amesite (AMST)	\$15M	\$109M	0%	+4%	+4%
<i>Provides an AI-driven platform for developing online learning products.</i>					
FinTech Acquisition IV (FTIVU)	\$200M	\$275M	0%	+3%	+3%
<i>Fifth blank check company formed by management of The Bancorp to acquire a fintech business.</i>					
Graybug Vision (GRAY)	\$90M	\$353M	0%	+3%	+3%
<i>Phase 2 biotech developing long-acting formulations of drugs for ocular diseases.</i>					
Falcon Capital Acq. (FCACU)	\$300M	\$375M	0%	+4%	+2%
<i>Blank check company formed by Ariliam Group and Eagle Equity Partners targeting a media business.</i>					

Corsair Gaming (CRSR)	\$238M	\$1,680M	0%	-16%	+1%
<i>Designs and supplies personal computer and gaming hardware components.</i>					
ArcLight Clean Transition (ACTCU)	\$250M	\$313M	0%	+0%	+0%
<i>Blank check company formed by ArcLight Capital Partners targeting a renewable energy business.</i>					
Vector Acquisition (VACQU)	\$300M	\$375M	0%	+0%	+0%
<i>Blank check company formed by Vector Capital targeting the tech industry.</i>					
Peridot Acquisition (PDACU)	\$300M	\$375M	0%	-1%	+0%
<i>Blank check company formed by Carnelian Energy Capital targeting businesses with a positive environmental impact.</i>					
Aspirational Consumer (ASPL.U)	\$225M	\$281M	0%	+0%	+0%
<i>Blank check company formed by executives at LVMH and L Catterton targeting a lifestyle business.</i>					
VPC Impact Acquisition (VIHAU)	\$200M	\$250M	0%	+0%	+0%
<i>Blank check company formed by Victory Park Capital to acquire a fintech business.</i>					
PMV Consumer Acq. (PMVC.U)	\$175M	\$219M	0%	+0%	+0%
<i>Blank check company formed by the Gabelli Group targeting the consumer industry.</i>					
Greenwich LifeSciences (GLSI)	\$7M	\$78M	-28%	-13%	-13%
<i>Phase 3 biotech developing immunotherapies for breast cancer.</i>					
VIA optronics (VIAO)	\$94M	\$338M	-6%	-32%	-32%
<i>German provider of sunlight readable, ultrathin display and touch solutions.</i>					

Eight IPOs and 18 SPACs submitted initial filings. Chinese discount retailer Miniso Group Holding ([MNSO](#)) filed to raise an estimated \$1 billion. Australian Phase 3 biotech Opthea ([OPT](#)) filed to raise \$150 million. Solar panel mounting manufacturer Array Technologies ([ARRY](#)), medical device companies Spinal Elements Holdings ([SPEL](#)) and Eargo (EAR), and biotechs Praxis Precision Medicines (PRAX) and Aligos Therapeutics (ALGS) all filed to raise \$100 million. Ophthalmology biotech Tarsus Pharmaceuticals (TARS) filed to raise \$86 million.

Pacific Century and Thiel Capital's SPAC Bridgetown Holdings (BTWNU) filed to raise \$500 million. Pine Island Capital Partner's SPAC Pine Island Acquisition (PIPP.U), Michael Klein's fifth SPAC Churchill Capital Corp V (CCV.U), and travel SPAC Altitude Acquisition (ALTUU) all filed to raise \$300 million. SPAC Supernova Partners Acquisition (SPNV.U) and digital media SPAC Tekkorp Digital Acquisition ([TEKKU](#)) both filed to raise \$300 million. Riverstone's third SPAC Decarbonization Plus Acquisition (DCRBU) and KSH Capital's SPAC Jack Creek Investment (JCICU) both filed to raise \$300 million. Sustainability SPAC Spring Valley Acquisition (SVSVU) filed to raise \$250 million. Venture firm's tech SPAC 10X Capital Venture Acquisition (VCVCU) filed to raise \$220 million.

Boston Omaha's SPAC Yellowstone Acquisition (YSU), and aviation SPAC Genesis Park Acquisition both filed to raise \$200 million. Eric Rosenfeld and David Sgro's SPAC Legato Merger (LEGOU) filed to raise \$175 million, while SPAC Motion Acquisition (MOTNU) filed to raise \$130 million. Mallard Capital's SPAC Mallard Acquisition ([MACUU](#)) filed to raise \$100 million. SPAC 5:01 Acquisition ([FVAM](#)) filed to raise \$80 million. Sports and entertainment SPAC Bull Horn Holdings (BHSEU) and growth-focused SPAC New Beginnings Acq. (NBA.U) both filed to raise \$75 million.

24 Filings During the Week of September 21st, 2020

Issuer Business	Deal Size	Sector	Lead Underwriter
10X Capital Venture Acq. (VCVCU)	\$220M	SPAC	Cantor Fitz.
<i>Blank check company formed by 10X Capital targeting a tech-enabled business.</i>			
5:01 Acquisition (FVAM)	\$80M	SPAC	BofA
<i>Blank check company formed by 5AM Ventures targeting businesses in the biotech industry.</i>			
Altitude Acquisition (ALTUU)	\$300M	SPAC	Cantor Fitz.
<i>Blank check company targeting the travel industry.</i>			
Eargo (EAR)	\$100M	Health Care	JP Morgan
<i>Manufactures differentiated hearing aid systems.</i>			
Genesis Park Acquisition (GNPK.U)	\$200M	SPAC	Jefferies
<i>Blank check company formed by Genesis Park targeting the aviation industry.</i>			
Motion Acquisition (MOTNU)	\$130M	SPAC	Barclays
<i>Blank check company formed by the former CEO of Fleetmatics targeting the telematics industry.</i>			
Praxis (PRAX)	\$100M	Health Care	Cowen
<i>Phase 2 biotech developing genetic therapies for brain disorders.</i>			
Spring Valley Acquisition (SVSVU)	\$250M	SPAC	Cowen
<i>Blank check company formed by Pearl Energy Investment Management targeting the sustainability industry.</i>			
Supernova Partners Acq. (SPNV.U)	\$300M	SPAC	JP Morgan
<i>Blank check company formed by the co-founder of Zillow and other entrepreneur and investment veterans.</i>			
Tekkorp Digital Acq. (TEKKU)	\$300M	SPAC	Jefferies
<i>Blank check company formed by Tekkorp Capital targeting the digital media and leisure industries.</i>			

Yellowstone Acquisition (YSU)	\$200M	SPAC	Wells Fargo
<i>Blank check company formed by Boston Omaha Corp. targeting the financial and real estate sectors.</i>			
Opthea (OPT)	\$150M	Health Care	Citi
<i>Australian Phase 3 biotech developing VEGF inhibitors for wet AMD.</i>			
Bridgetown Holdings (BTWNU)	\$500M	SPAC	UBS
<i>Blank check company formed by Pacific Century and Thiel Capital targeting "new economy" sectors in Southeast Asia.</i>			
Legato Merger (LEGOU)	\$175M	SPAC	EarlyBird
<i>Blank check company formed by SPAC veterans Eric Rosenfeld and David Sgro targeting an industrial business.</i>			
Miniso Group Holding (MNSO)	\$1,000M	Consumer Discretionary	Goldman
<i>Chinese discount retailer selling branded lifestyle and home goods.</i>			
Pine Island Acquisition (PIPP.U)	\$300M	SPAC	Citi
<i>Blank check company formed by Pine Island Capital Partners targeting the defense industry.</i>			
Spinal Elements Holdings (SPEL)	\$100M	Health Care	Credit Suisse
<i>Develops medical devices for minimally invasive spinal surgery procedures.</i>			
Array Technologies (ARRY)	\$100M	Industrials	Goldman
<i>Manufactures ground-mounting systems used in solar energy projects.</i>			
Churchill Capital Corp V (CCV.U)	\$300M	SPAC	Citi
<i>Fifth blank check company founded by dealmaker and former Citi executive Michael Klein.</i>			
Decarbonization Plus Acq. (DCRBU)	\$300M	SPAC	Citi
<i>Third blank check company formed by Riverstone targeting businesses advancing global decarbonization.</i>			
Bull Horn Holdings (BHSEU)	\$75M	SPAC	Imperial Capital

Blank check company targeting leading sports, entertainment, and brand companies.

Jack Creek Investment (JCICU)	\$300M	SPAC	JP Morgan
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Blank check company formed by KSH Capital targeting a food or consumer business.

Mallard Acquisition (MACUU)	\$100M	SPAC	Chardan
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Blank check company formed by Mallard Capital targeting an industrials business.

New Beginnings Acq. (NBA.U)	\$75M	SPAC	Ladenburg
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Blank check company formed by the co-founder of Crescent Heights and an executive from Alliant Insurance Services.

IPO Market Snapshot

The Renaissance IPO Indices are market cap weighted baskets of newly public companies. As of 9/17/20, the Renaissance IPO Index was up 59.7% year-to-date, while the S&P 500 was up 0.5%. Renaissance Capital's IPO ETF ([IPO](#)) tracks the index, and top ETF holdings include Zoom Video ([ZM](#)) and Uber ([UBER](#)). The Renaissance International IPO Index was up 30.8% year-to-date, while the ACWX was down 6.8%. Renaissance Capital's International IPO ETF ([IPOS](#)) tracks the index, and top ETF holdings include Xiaomi and Meituan-Dianping.

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Editor's Note: The summary bullets for this article were chosen by Seeking Alpha editors.

This article was written by



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U.S. IPO Weekly Recap: The September IPO Market Kicks Off With A Quiet Week

Sep. 06, 2020 1:44 PM ET | WGS, LMND, TNGX...



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Summary

- There were no IPOs this past week.
- Five SPACs went public ahead of the post-Labor Day rush.
- 14 IPOs and 11 SPACs submitted initial filings.

While there were no IPOs this past week, five SPACs went public ahead of the post-Labor Day rush.

Casdin Capital and Corvex Management's SPAC CM Life Sciences (CMLF) upsized to raise \$385 million. Led by the firms' founders, Eli Casdin and Keith Meister, the company plans to target the life science industry, specifically businesses in the life science tools, synthetic biology, and diagnostics fields.

Tech SPAC Tailwind Acquisition (TWND.U) raised \$300 million. The company was formed by Chairman Philip Krim, co-founder and CEO of Casper ([CSPR](#)), which is down 24% from its February IPO. Venture capitalist Chris Hollod serves as CEO, and Tengram Capital Partners co-founder Matt Eby serves as CFO.

INSU Acquisition II (INAQU), the second blank check company formed by Cohen & Company and targeting the insurance industry, upsized to raise \$200 million. Chairman Daniel Cohen and CEO John Butler serve in the same roles for Insurance Acquisition (INSU; +33% from IPO), which recently announced a merger agreement with Shift Technologies.

Boxer Capital's warrantless SPAC BCTG Acquisition (BCTG) upsized to raise \$145 million. The company is led by Boxer Capital co-founders Aaron Davis and Christopher Fuglesang, and plans to leverage its management team's experience and target a business in the biotechnology industry.

Life sciences SPAC HighCape Capital Acquisition (CAPAU) raised \$100 million. Led by HighCape Capital co-founders, the company plans target the life sciences industry, specifically therapeutics, devices, diagnostics, medical information technology, agrisciences, and animal health.

5 IPOs During the Week of August 31st, 2020

Issuer <i>Business</i>	Deal Size	Market Cap at IPO	Price vs. Midpoint	First Day Return	Return at 09/04
CM Life Sciences	\$385M	\$473M	0%	+3%	+3%
<i>Blank check company formed by Casdin Capital and Corvex Management targeting a life science business.</i>					
INSU Acquisition II	\$200M	\$266M	0%	+3%	+3%
<i>Second blank check company formed by Cohen & Company and targeting the insurance industry.</i>					
BCTG Acquisition	\$145M	\$181M	0%	+2%	+3%
<i>Blank check company formed by Boxer Capital targeting the biotech industry.</i>					
HighCape Capital Acq.	\$100M	\$129M	0%	+2%	+2%
<i>Blank check company formed by HighCape Capital targeting the life sciences industry.</i>					
Tailwind Acquisition	\$300M	\$375M	0%	+0%	+0%
<i>Blank check company formed by Casper CEO Philip Krim targeting a technology business.</i>					

14 IPOs and 11 SPACs submitted initial filings.

SMB services provider Thryv Holdings ([THRY](#)) filed for a direct listing on the Nasdaq. Rare disease biotech Orphazyme ([ORPH](#)) filed to raise \$115 million. Avocado supplier Mission Produce ([AVO](#)), LNG services provider Hygo Energy Transition ([HYGO](#)), gene therapy biotech Taysha Gene Therapies ([TSHA](#)), optical bonding company VIA optronics ([VIAO](#)), and oncology biotechs PMV Pharmaceuticals ([PMVP](#)) and Prelude Therapeutics ([PRLD](#)) all filed to raise \$100 million. Ocular disease biotech Graybar Vision ([GRAY](#)) and medical device maker Pulmonx ([LUNG](#)) filed to raise \$86 million. Plant-based beverage manufacturer Laird Superfood ([LSF](#)) filed to raise \$40 million. Chinese real estate holding company Sancai Holding Group ([SCIT](#)) filed to raise \$30 million. Chinese agriculture services provider Green Grass ([QQCY](#)) filed to raise \$24 million, and micro-cap oncology biotech Lixte Biotechnology ([LIXT](#)) filed to raise \$11 million.

Energy SPAC Bluescape Opportunities Acquisition ([BOAC](#)) filed to raise \$700 million. Tech SPAC Reinvent Technology Partners ([RTPU](#)) filed to raise \$600 million. TPG's two SPACs TPG Pace Tech Opportunities ([PACE.U](#)) and TPG Pace Beneficial Finance ([TPGY.U](#)) filed to raise \$450 million and \$350 million, respectively. Private equity firm's SPAC Peridot Acquisition ([PDAC.U](#)), media SPAC Falcon Capital Acquisition ([FCACU](#)), and renewable energy SPAC ArcLight Clean Transition ([ACTCU](#)) all filed to raise \$300 million. ACON Investments' SPAC ACON S2 Acquisition ([STWOU](#)) filed to raise \$250 million. Luxury brand SPAC Aspirational Consumer Lifestyle Corp. ([ASCL.RC](#)) and Oaktree's Oaktree Acquisition II ([OACBU](#)) both filed to raise \$225 million. Healthcare SPAC Blue Water Acquisition ([BLUWU](#)) filed to raise \$50 million.

25 Filings During the Week of August 31st, 2020

Issuer Business	Deal Size	Sector	Lead Underwriter
ArcLight Clean Transition	\$300M	SPAC	Citi
<i>Blank check company formed by ArcLight Capital Partners targeting a renewable energy business.</i>			
Aspirational Consumer	\$225M	SPAC	Credit Suisse
<i>Blank check company formed by executives at LVMH and L Catterton targeting a lifestyle business.</i>			
Graybug Vision	\$86M	Health Care	SVB Leerink
<i>Phase 2 biotech developing transformative medicines for ocular diseases.</i>			
Mission Produce	\$100M	Consumer Staples	BofA
<i>Leading supplier of fresh avocados.</i>			
Orphazyme	\$115M	Health Care	BofA
<i>Danish late-stage biotech developing protein therapies for rare neurodegenerative diseases.</i>			
Peridot Acquisition	\$300M	SPAC	UBS
<i>Blank check company formed by Carnelian targeting business with Mitigation and Adaptation principles.</i>			
PMV Pharmaceuticals	\$100M	Health Care	Goldman
<i>Early stage biotech developing targeted therapies for cancer.</i>			
Prelude Therapeutics	\$100M	Health Care	Morgan Stanley
<i>Phase 1 biotech developing small molecule therapies for cancer.</i>			
Pulmonx	\$86M	Health Care	BofA
<i>Makes minimally invasive medical devices for emphysema.</i>			
Sancai Holding Group	\$30M	Real Estate	Univest Securities
<i>Real estate holding company in China.</i>			

TPG Pace Beneficial Fin.	\$350M	SPAC	Deutsche Bank
<i>Blank check company formed by TPG Pace Group targeting a business with a strong ESG profile.</i>			
TPG Pace Tech	\$450M	SPAC	Deutsche Bank
<i>Blank check company formed by TPG Pace Group targeting the tech industry.</i>			
VIA optronics	\$100M	Technology	Berenberg
<i>German provider of sunlight readable, ultrathin display and touch solutions.</i>			
Blue Water Acquisition	\$50M	SPAC	Maxim
<i>Blank check company formed by an industry veteran targeting a healthcare business.</i>			
Falcon Capital Acq.	\$300M	SPAC	Goldman
<i>Blank check company formed by Ariliam Group and Eagle Equity Partners targeting a media business.</i>			
Green Grass	\$24M	Industrials	---
<i>Provides specialty farming and agriculture services in China.</i>			
Lixte Biotechnology	\$11M	Health Care	WestPark Capital
<i>Phase 2 biotech using biomarker technology to develop protein inhibitors for cancer.</i>			
Bluescape Opp. Acq.	\$700M	SPAC	Citi
<i>Blank check company formed by Bluescape Energy Partners targeting the energy and industrial sectors.</i>			
Taysha Gene Therapies	\$100M	Health Care	Goldman
<i>Early stage biotech developing AAV-based gene therapies for CNS disorders.</i>			
Thryv Holdings	\$272M	Technology	
<i>Provides marketing solutions and SaaS customer experience tools to SMBs.</i>			
ACON S2 Acquisition	\$250M	SPAC	Deutsche Bank
<i>Blank check company formed by ACON Investments targeting a sustainable business.</i>			

Hygo Energy Transition	\$100M	Energy	Morgan Stanley
<i>Provides integrated downstream liquefied natural gas solutions in Brazil.</i>			
Laird Superfood	\$40M	Consumer Staples	Canaccord
<i>Manufactures plant-based packaged beverage products.</i>			
Oaktree Acquisition II	\$225M	SPAC	Deutsche Bank
<i>Second blank check company led by the head of Oaktree Capital's Value Equities group.</i>			
Reinvent Tech Partners	\$600M	SPAC	Morgan Stanley
<i>Blank check company formed by LinkedIn co-founder Reid Hoffman and Zynga founder Mark Pincus targeting the tech sector.</i>			

IPO Market Snapshot

The Renaissance IPO Indices are market cap weighted baskets of newly public companies. As of 9/3/20, the Renaissance IPO Index was up 54.8% year-to-date, while the S&P 500 was up 6.9%. Renaissance Capital's IPO ETF ([IPO](#)) tracks the index, and top ETF holdings include Zoom Video ([ZM](#)) and Uber ([UBER](#)). The Renaissance International IPO Index was up 38.6% year-to-date, while the ACWX was down 4.2%. Renaissance Capital's International IPO ETF ([IPOS](#)) tracks the index, and top ETF holdings include Xiaomi and Meituan-Dianping.

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Editor's Note: The summary bullets for this article were chosen by Seeking Alpha editors.

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Wall Street Breakfast: The Week Ahead

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Investors head into the last week of the quarter with a slightly more cautious positioning as recession fears have jumped to the forefront again. A slow calendar of economic releases means that traders may stew further on Federal Reserve Chairman Jerome Powell's Congressional testimony in which he signaled more interest rate hikes are ahead.

Meanwhile, the release of the results for the Federal Reserve's annual stress tests will be a major focus in the banking sector. [Seeking Alpha analyst Stephen Simpson](#) said the bigger changes for banks are likely to come later, as the Federal Reserve contemplates new rules tied to Basel III Endgame and the failures of Silicon Valley Bank and First Republic. Simpson noted that while the largest banks seem less vulnerable, capital requirement changes could meaningfully impact the profitability of regional banks, with uncertainty likely to weigh on bank stocks in general for a while longer.

Earnings spotlight: Monday, June 26 - Carnival ([CCL](#)).

Earnings spotlight: Tuesday, June 27 - Walgreens Boots Alliance ([WBA](#)), Manchester United ([MANU](#)), and Jefferies Financial ([JEF](#)).

Earnings spotlight: Wednesday, June 28 - Micron Technology ([MU](#)), General Mills ([GIS](#)), and BlackBerry ([BB](#)).

Earnings spotlight: Thursday, June 29 - Nike ([NKE](#)), Paychex ([PAYX](#)), McCormick ([MKC](#)), and Simply Good Foods ([SMPL](#)).

Earnings spotlight: Friday, June 30 - Constellation Brands ([STZ](#)).

Volatility watch: Short interest on both ImmunityBio (NASDAQ:[IBRX](#)) is at 18.29% and Kura Sushi (NASDAQ:[KRUS](#)) is at 22.14% of total float. Options trading volume is elevated on Vroom ([VRM](#)) and FibroGen ([FGEN](#)) once again. Virgin Galactic ([SPCE](#)) continues to see huge price swings as longs and shorts battle it out.

Quant ratings: Stocks with recent quant rating changes include Shopify ([SHOP](#)) to Strong Buy from Hold, Amazon ([AMZN](#)) to Strong Buy from Hold, and Blue Apron ([APRN](#)) to Strong Sell from Hold. See the stocks with the very highest rated [Seeking Alpha Quant Ratings](#).

Dividend watch: Companies forecast to boost their quarterly payouts include Korn Ferry ([KFY](#)) to \$0.17 from \$0.15, Worthington Industries ([WOR](#)) to \$0.34 from \$0.31, Micron ([MU](#)) to \$0.12 from \$0.115, and Lindsay ([LNN](#)) to \$0.35 from \$0.34. Stocks with ex-dividend dates for their upcoming dividend payments include ConocoPhillips ([COP](#)) and Mondelez International ([MDLZ](#)). [See Seeking Alpha's list of Quick Dividend Stock Picks.](#)

Corporate events: The four-day Snowflake (NYSE:[SNOW](#)) Summit 2023 will attract attention next week, with the company expected to give investors a deeper look at its latest innovations, use cases, and thought leadership. The summit will include a fireside chat on generative AI with Nvidia ([NVDA](#)) CEO Jensen Huang. Delta Air Lines ([DAL](#)) will webcast the company's Investor Day presentation on June 27.

Also, on June 27, Casey's General Stores ([CASY](#)) will outline an updated three-year strategic plan at an investor event. On June 28, Taysha Gene Therapies ([TSHA](#)) will host a virtual R&D Day to discuss updates on TSHA-120 and Canadian Pacific Kansas City (NYSE:[CP](#)) will webcast its Investor Day presentations in Kansas City. AMC Entertainment ([AMC](#)) and AMC Preferred Equity units ([APE](#)) will be on watch during the last two days of the week as a two-day settlement hearing takes place in Delaware's Court of Chancery. Both stocks have swung wildly off developments in the stock conversion case. See a detailed list of key events for next week in [Seeking Alpha's Catalyst Watch](#).

Investor conferences: The conference schedule for the week ahead is light, with the H.C. Wainwright 4th Annual Neuropsychiatry Virtual Conference, and UBS Healthcare Services Conference standing out as the most notable. The Jefferies Space Summit will include appearances by Terran Orbital (NYSE:[LLAP](#)), MDA ([MDA:CA](#)), Sidus Space ([SIDU](#)), BlackSky Technology ([BKSY](#)), and Redwire (NYSE:[RDW](#)).

This article was written by



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TSHA Earnings Date

Earnings announcement* for TSHA:

Our vendor, Zacks Investment Research, hasn't provided us with the upcoming earnings report date.

Latest Press Release

Earnings Per Share

ESTIMATED REPORTED

4Q'22 1Q'23 2Q'23 3Q'23 4Q'23 1Q'24 2Q'24 3Q'24

-1.25 -1.075 -0.5 -0.250

EPS

ESTIMATED EPS 1.00

REPORTED EPS: 1.00

Quarterly Earnings Surprise Amount

Fiscal Quarter End	Date Reported	Earnings Per Share*	Consensus EPS* Forecast	% Surprise
Sep 2023	11/14/2023	-0.13	-0.13	0
Jun 2023	08/14/2023	-0.38	-0.31	-22.58
Mar 2023	05/11/2023	-0.28	-0.39	28.21
Dec 2022	03/28/2023	-0.99	-0.41	-141.46

Yearly Earnings Forecast

Fiscal Year End	Consensus EPS* Forecast	High EPS* Forecast	Low EPS* Forecast	Number Of Estimates	Over The Last 4 Weeks Number Of Revisions - Up	Over The Last 4 Weeks Number Of Revisions - Down
Dec 2023	-0.69	-0.45	-0.82	3	0	0
Dec 2024	-0.45	-0.33	-0.61	4	0	0
Dec 2025	-0.38	-0.38	-0.38	1	0	0
Dec 2026	-0.39	-0.39	-0.39	1	0	0

Quarterly Earnings Forecast

Fiscal Quarter End	Consensus EPS* Forecast	High EPS* Forecast	Low EPS* Forecast	Number Of Estimates	Over The Last 4 Weeks Up	Over The Last 4 Weeks Number Of Revisions - Up	Over The Last 4 Weeks Number Of Revisions - Down
Dec 2023-0.1	-0.09	-0.1	2	0	0	0	0
Mar 2024-0.1	-0.1	-0.1	1	0	0	0	0
Jun 2024-0.1	-0.1	-0.1	1	0	0	0	0
Sep 2024-0.1	-0.1	-0.1	1	0	0	0	0
Dec 2024-0.11	-0.11	-0.11	1	0	0	0	0

Change in Consensus

FISCAL YEAR END DEC 2023 FISCAL QUARTER END DEC 2023
 1 Month Ago 1 Week Ago Current
 -1-0.50

Number of Estimates Changed

Fiscal QE Dec 2023 Down	0
Fiscal QE Dec 2023 Up	0
Fiscal YR Dec 2023 Down	0
Fiscal YR Dec 2023 Up	0

Estimate Momentum measures change in analyst sentiment over time and may be an indicator of future price movements. The Change in Consensus chart shows the current, 1 week ago, and 1 month ago consensus earnings per share (EPS*) forecasts. For the fiscal quarter ending Dec 2023 , the consensus EPS* forecast has remained the same over the past week at -0.1 and remained the same over the past month at -0.1. none raised and none lowered their forecast. For the fiscal year ending Dec 2023 , the consensus EPS* forecast has remained the same over the past week at -0.69 and remained the same over the past month at -0.69 . none raised and none lowered their forecast.

TSHA Price/Earnings & PEG Ratios

Price/Earnings Ratio

	label	value
2022 Actual		-0.47
2023 Estimates		-2.58
2024 Estimates		-3.96
2025 Estimates		-4.68

Forecast P/E Growth Rates

	label	value
Growth 2023		81.75
Growth 2024		35.51
P/E Ratios 2023		-2.58
P/E Ratios 2024		-3.96

PEG Ratio

	label	value
Forecast 12 Month Forward PEG Ratio		0

TSHA Short Interest

Settlement Date	Short Interest	Avg. Daily Share Volume	Days To Cover
12/15/2023	17,126,628	2,116,197	8.093116
11/30/2023	16,157,351	2,024,873	7.979439
11/15/2023	13,954,891	3,579,061	3.899037
10/31/2023	9,760,464	1,690,697	5.773042
10/13/2023	10,015,852	1,350,412	7.416886
09/29/2023	10,668,528	1,638,765	6.510102
09/15/2023	9,927,037	2,951,576	3.3633
08/31/2023	7,357,091	5,594,248	1.315117
08/15/2023	732,065	17,977,074	1

Settlement Date	Short Interest	Avg. Daily Share Volume	Days To Cover
07/31/2023	1,097,175	149,452	7.34132
07/14/2023	1,122,621	186,606	6.015996
06/30/2023	1,279,590	478,147	2.676144
06/15/2023	1,425,240	193,452	7.367409
05/31/2023	1,225,447	252,159	4.859819
05/15/2023	1,189,446	258,033	4.609666
04/28/2023	1,725,104	375,478	4.594421
04/14/2023	1,632,088	636,826	2.562848
03/31/2023	1,927,280	582,353	3.30947
03/15/2023	2,355,880	243,506	9.674833
02/28/2023	2,668,498	357,245	7.469658
02/15/2023	2,234,526	1,078,469	2.071943
01/31/2023	2,178,643	540,520	4.030643
01/13/2023	1,716,412	380,857	4.50671

TSIA Institutional Holdings

Ownership Summary

Label	Value
Institutional Ownership	69.18%
Total Shares Outstanding (millions)	187
Total Value of Holdings (millions)	\$230

Active Positions

Active Positions	Holders	Shares
Increased Positions	47	120,026,300
Decreased Positions	17	3,767,418
Held Positions	8	5,538,669
Total Institutional Shares	72	129,332,387

New and Sold Out Positions

Active Positions	Holders	Shares
New Positions	33	89,609,268
Sold Out Positions	11	3,686,796

TSHA Insider Activity

Number of Insider Trades

Insider Trade	3 Months	12 Months
Number of Open Market Buys	4	14
Number of Sells	0	7
Total Insider Trades	4	21

Number of Insider Shares Traded

Insider Trade	3 Months	12 Months
Number of Shares Bought	2,738,718	20,869,935
Number of Shares Sold	0	582,095
Total Shares Traded	2,738,718	21,452,030
Net Activity	2,738,718	20,287,840

TSHA Revenue EPS

Fiscal Quarter:	2020 (Fiscal Year)	2019 (Fiscal Year)	2018 (Fiscal Year)
September			
Revenue	N/A		

Fiscal Quarter:	2020 (Fiscal Year)	2019 (Fiscal Year)	2018 (Fiscal Year)
EPS	(\$1.28) (09/30/2020)		
Dividends	N/A		
December (FYE)			
Revenue	N/A		
EPS	\$0.33 (12/31/2020)		
Dividends	N/A		
December (FYE)			
Revenue			
EPS			
Dividends			
September			
Revenue			
EPS			
Dividends			
Totals			
Revenue	N/A	N/A	N/A
EPS	(\$0.95)	N/A	N/A
Dividends	N/A	N/A	N/A

Taysha Gene Therapies, Inc. Common Stock (TSHA) Revenue EPS

TSHA Revenue EPS

Fiscal Quarter:	2023 (Fiscal Year)	2022 (Fiscal Year)	2021 (Fiscal Year)
March			
Revenue	\$4(m)	\$0	N/A
EPS	(\$0.28) (03/31/2023)	(\$1.32) (03/31/2022)	(\$0.87) (03/31/2021)
Dividends	N/A	N/A	N/A
June			
Revenue	\$2(m)	\$0	N/A
EPS	(\$0.38) (06/30/2023)	(\$0.84) (06/30/2022)	(\$1.09) (06/30/2021)
Dividends	N/A	N/A	N/A
September			
Revenue	\$4(m)	\$0	N/A
EPS	(\$1.22) (09/30/2023)	(\$0.63) (09/30/2022)	(\$1.35) (09/30/2021)
Dividends	N/A	N/A	N/A
December (FYE)			
Revenue		\$2(m)	\$0

Fiscal Quarter:	2023 (Fiscal Year)	2022 (Fiscal Year)	2021 (Fiscal Year)
EPS		(\$0.99) (12/31/2022)	(\$1.33) (12/31/2021)
Dividends		N/A	N/A
Totals			
Revenue	\$11(m)	\$2(m)	N/A
EPS	(\$1.88)	(\$3.78)	(\$4.64)
Dividends	N/A	N/A	N/A

Taysha Gene Therapies, Inc. Common Stock (TSHA) Revenue EPS

Healthcare

IPO News

Taysha Gene Therapies on deck for IPO

Sep. 03, 2020 12:21 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | TSHA | By: Douglas W. House, SA News Editor

- **Taysha Gene Therapies (TSHA)** has filed a [preliminary prospectus](#) for a \$100M IPO.
- The Dallas, TX-based biotech develops adeno-associated virus (AAV)-based gene therapies for monogenic (mutations in a single gene) central nervous system diseases.
- Lead candidate is TSHA-101 for the potential treatment of [GM2 gangliosidosis](#), a rare inherited lysosomal storage disorder characterized by the progressive destruction of nerve cells in the brain and spinal cord. A Phase 1/2 study in Canada should launch by year-end.
- **2020 Financials** (6 mo.): Operating Expenses: \$9.6M (+773%); Net Loss: (\$26.7M) (-999%); Cash Burn: (\$4.2M).

PROGRAM	INDICATION	PRECLINICAL	IND-ENABLING	PHASE 1/2	PIVOTAL	RIGHTS
NEURODEGENERATIVE DISEASES						
TSHA-101 GRT	GM2 Gangliosidosis			Clinical expected in 2020		
TSHA-118/ ABD-202 GRT	CUN1			Currently open IND		
TSHA-104 GRT	SURF1 Deficiency			Clinical expected in 2021		
TSHA-112 GRT/miRNA	APBD					
TSHA-111 GRT/miRNA	LaFora					
TSHA-113 miRNA	Tauopathies					
TSHA-115 miRNA	GSDs					
NEURODEVELOPMENTAL DISORDERS						
TSHA-102 Regulated GRT	Rett Syndrome			Clinical expected in 2021		
TSHA-106 shRNA	Angelman Syndrome					
TSHA-114 GRT	Fragile X Syndrome					
TSHA-116 shRNA	Prader-Willi Syndrome					
TSHA-117 Regulated GRT	FOXP1					
TSHA-107 GRT	Undisclosed Target					
TSHA-108 GRT	Undisclosed Target					
TSHA-109 GRT	Undisclosed Target					
GENETIC EPILEPSIES						
TSHA-103 GRT	SLC6A1			Clinical expected in 2021		
TSHA-105 GRT	SLC13A5					
TSHA-110 GRT	KCNQ2*					

*Option rights

** Taysha has exclusive options to acquire an additional four programs from UT Southwestern
GRT: Gene replacement therapy miRNA: microRNA shRNA: short hairpin RNA

[Healthcare](#)[IPO News](#)

Taysha Gene Therapies sets IPO terms, seeks to raise ~\$131M

Sep. 17, 2020 1:24 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | TSHA | By: Vandana Singh, SA News Editor

- Taysha Gene Therapies ([TSHA](#)) [files to sell](#) ~6.8M shares in the range of \$18 to \$20 and seeks to raise up to \$131.6M.
- With ~34.5M shares outstanding after the IPO, the company could be valued at up to \$690.4M.
- Initially, the company [filed for \\$100M IPO](#).

[Healthcare](#)[IPO News](#)

Taysha Gene Therapies prices upsized IPO at \$20/share

Sep. 24, 2020 7:54 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Nilofer Shaikh, SA News Editor
| 2 Comments

- Taysha Gene Therapies (NASDAQ:TSHA) has priced an upsized initial public offering of ~7.9M (from 6.8M) shares at \$20/share, at the high end of the prior expected range of \$18 - \$20.
- The shares to kick-off trading today on the Nasdaq Global Select Market.
- Offering is expected to close on September 28, 2020.
- Underwriters' over-allotment is an additional 1,180,434 shares.
- The company raised \$157M, 26% more in proceeds than expected.
- Previously: [Taysha Gene Therapies sets IPO terms, seeks to raise ~\\$131M](#) (Sept. 17)

[Healthcare](#)[M&A](#)

Taysha Gene teams up with Invitae for rapid testing and identification of patients with CNS disease

Oct. 06, 2020 8:32 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock, NVTA Stock** | By: Meghavi Singh, SA News Editor | 10 Comments

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) announces partnership with Invitae (NYSE:[NVTA](#)) to support the latter's Detect Lysosomal Storage Diseases (Detect LSDs) and Behind the Seizure programs.
- The Detect LSDs program enables the rapid diagnosis of lysosomal storage disorders (LSDs) like Gaucher disease, including GM2 gangliosidosis containing different types of lipid storage disorders.
- The Behind the Seizure program supports faster diagnosis for children with epilepsy.
- The study will enroll individuals suspected of having an LSD or epilepsy and will gain access to genetic testing and counseling.
- The Detect LSDs program will help identify individuals eligible for Taysha's study evaluating TSHA-101 in patients with GM2 gangliosidosis, expected to enter the clinic later this year.
- The Behind the Seizure program will enable identification of patients having underlying seizure phenotype, and rapid enrollment into clinical trials.

Healthcare

Taysha nabs Orphan Drug and Rare Pediatric status for Rett syndrome gene therapy

Oct. 14, 2020 9:19 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Vandana Singh, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has received rare pediatric disease and orphan drug designation from the FDA for TSHA-102, an AAV9-based gene therapy for the treatment of Rett syndrome. Submission of an Investigational New Drug application for TSHA-102 to the FDA, is expected in 2021.
- Among the benefits of Orphan Drug status in the U.S. is a seven-year period of market exclusivity for the indication, if approved.
- Rare Pediatric Disease designation provides for the issuance of a priority review voucher following FDA approval. The voucher can be used for accelerated approval of a future application or it can be sold to a third party
- Rett syndrome caused by mutations in the MECP2 gene, is a rare genetic neurological and developmental disorder that affects the way the brain develops, causing a progressive loss of motor skills and speech, and primarily affects girls.
- TSHA-102 is designed to deliver a healthy version of the MECP2 gene as well as the miRNA-Responsive Auto-Regulatory Element, miRARE, platform technology to control the level of MECP2 expression.

Healthcare

Taysha's gene therapy nabs Orphan Drug and Rare Pediatric status for inherited neurological disorder

Oct. 27, 2020 8:23 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Vandana Singh, SA News Editor

- The FDA has designated both rare pediatric disease and Orphan Drug tags to Taysha Gene Therapies' (NASDAQ:TSHA) TSHA-104, an AAV9-based gene therapy in development for SURF1-associated Leigh syndrome, a severe neurological disorder characterized by the progressive loss of mental and movement abilities. It is usually fatal within the first few years of life.
- The company expects to submit an Investigational New Drug application for TSHA-104 in 2021.
- Among the benefits of Orphan Drug status in the U.S. is a seven-year period of market exclusivity for the indication, if approved.
- Rare Pediatric Disease designation provides for the issuance of a priority review voucher following FDA approval. The voucher can be used for accelerated approval of a future application or it can be sold to a third party

Healthcare

Taysha Gene Therapies nabs Orphan Drug and Rare Pediatric status for TSHA-103 in autosomal genetic disorder

Dec. 03, 2020 7:22 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has received both orphan drug and rare pediatric disease designations from the FDA for TSHA-103, an AAV-9-based gene therapy in development for SLC6A1-related epilepsy.
- Among the benefits of Orphan Drug status in the U.S. is a seven-year period of market exclusivity for the indication, if approved.
- Rare Pediatric Disease status provides for the issuance of a rare pediatric disease priority review voucher following FDA approval. The voucher can be used for accelerated approval of a future application or it can be sold to a third party.
- SLC6A1 epilepsy is an autosomal dominant genetic disorder characterized by the loss of function of one copy of the SLC6A1 gene, with clinical manifestations of seizures, epilepsy, language impairment and intellectual disability.

Healthcare

Taysha on go with early-stage study with its gene therapy in inherited lysosomal storage disorder

Dec. 21, 2020 9:08 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Niloofar Shaikh, SA News Editor

| 1 Comment

- Health Canada signs off Taysha Gene Therapies's (NASDAQ:[TSHA](#)) open-label Phase 1/2 trial for TSHA-101, an investigational gene therapy for the treatment of GM2 gangliosidosis. Interim data is anticipated in 2021.
- Infantile GM2 gangliosidosis is a rare inherited lysosomal storage disorder characterized by the progressive destruction of nerve cells in the brain and spinal cord. Caused by defects in the HEXA or HEXB genes that encode the two subunits of the β -hexosaminidase A enzyme. These genetic defects result in progressive dysfunction of the central nervous system.
- The company says that TSHA-101 will be the first bicistronic vector to enter a first-in-human clinical study. It is designed to deliver both HEXA and HEXB transgenes within a single AAV9 vector construct.

Healthcare

Taysha teams up with AllStripes to advance gene therapy for rare disorder

Jan. 04, 2021 2:00 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

- AllStripes healthcare technology announces a multi-year collaboration with Taysha Gene Therapies (NASDAQ:TSHA) to focus on advancing the development of TSHA-104 for SURF1-associated Leigh syndrome.
- The AAV9-based gene therapy for the indication has been granted the rare pediatric disease and orphan drug designations by the FDA for which Taysha expects to submit an Investigational New Drug application in 2021.
- Per the agreement, AllStripes will use its platform that manages patient health histories to unify the SURF1-associated clinical data, allowing researchers to uncover insights into the natural history and burden of disease and better inform the development of clinical studies.
- The collaboration will “potentially accelerate the development of TSHA-104 in SURF1-associated Leigh syndrome,” says RA Session, II, President/CEO of Taysha. Nancy Yu, CEO of AllStripes added, “we are very pleased to empower patients and their families with an avenue to participate in research that will support the development path of TSHA-104.”
- Separately, *Bloomberg* says that Taysha has secured the orphan drug designation for a treatment targeting SLC13A5 Deficiency. The product candidate has a generic name Adeno-Associated Virus Serotype 9 (Aav9) Vector with Engineered Transgene Encoding the Human Slc13A5 Protein, the report added.
- In a Bullish thesis on the stock, Seeking Alpha contributor Donovan Jones said in September, the recently-IPOed company with its entire pipeline at a preclinical stage of development ‘may be more suited to long-term hold institutional investors.’

[Healthcare](#)[On the Move](#)

Taysha and UT Southwestern Medical Center launch innovation fund in gene therapy development push

Jan. 13, 2021 3:15 PM ET | [Taysha Gene Therapies, Inc. \(TSHA\) Stock](#) | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA +3.1%](#)) and UT Southwestern Medical Center (UTSW) launch an innovation fund to discover and develop novel gene therapy candidates and next-generation technologies for monogenic diseases of the central nervous system.
- Taysha will have an exclusive option on new programs and intellectual property associated with the research conducted under this agreement.
- The expanded collaboration between Taysha and UTSW is aimed at supporting the discovery of novel gene therapy candidates and next-generation technologies in new disease areas.
- Taysha's partnership with UTSW's gene therapy program has produced over 18 novel product candidates so far, including TSHA-101 in rare genetic disorder GM2 gangliosidosis and TSHA-118 in nervous system disorder CLN1, which are currently in clinical development.

[Healthcare](#)[On the Move](#)

Taysha Gene Therapies granted Orphan Drug designation for TSHA-105

Jan. 19, 2021 12:08 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- U.S. FDA awarded both rare pediatric disease and orphan drug designations to Taysha Gene Therapies (NASDAQ:[TSHA +10.7%](#)) for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy.
- "We are encouraged by the early evidence of TSHA-105's disease-modifying approach and believe these designations will help us potentially accelerate the development of this exciting program. We look forward to working with the FDA to make TSHA-105 available to patients as expeditiously as possible," president, founder & CEO RA Session II commented.
- SLC13A5 is a form of infantile epilepsy caused by mutations in the SLC13A5 gene.

Healthcare

Taysha Gene Therapies's new collaboration to target neurodevelopmental disorders

Mar. 09, 2021 3:30 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA +6.4%](#)) has announced a multi-year collaboration with Yong-Hui Jiang, a Professor and Chief of Medical Genetics at Yale University, to advance next-generation mini-gene payloads for AAV gene therapies targeted at neurodevelopmental disorders.
- Under the partnership, a team of researchers from Yale University will design mini-gene payloads for neurodevelopmental disorders including intellectual disability.
- The announcement follows the company's recent collaborations with Cleveland Clinic and UT Southwestern Gene Therapy Program ("UTSW") which is set to produce viral vector constructs incorporating mini-gene payloads for studies in in vivo and in vitro efficacy models.
- In January, Taysha Gene Therapies and UT Southwestern Medical Center announced the establishment of an innovation fund to advance gene therapy candidates and next-generation technologies targeting monogenic diseases of the CNS.

Healthcare

Taysha Gene acquires rights to AAV9 gene therapy program for severe neurodegenerative disease

Apr. 12, 2021 8:38 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) announces the acquisition of exclusive worldwide rights to a clinical-stage AAV9 gene therapy program, now known as TSHA-120, for the treatment of giant axonal neuropathy (GAN).
- GAN is a rare inherited genetic disorder that affects both the central and peripheral nervous systems and is caused by loss-of-function mutations in the gene coding for gigaxonin.
- Under the terms of the agreement, in exchange for TSHA-120 rights, the leading GAN patient advocacy group will receive an upfront payment of \$5.5M and will be eligible to receive milestones totaling up to \$19.3M, as well as a low, single-digit royalty on net sales upon commercialization of the product.
- Taysha expects to provide a regulatory and clinical update on TSHA-120, including data from the 3.5×10^{14} total vg cohort by year-end.
- Before the end of the year, TSHA intends to request an End-of-Phase meeting with the FDA and engage with EMA and Japanese regulatory agency to discuss the pathway for TSHA-120.

Healthcare

Taysha Gene price target raised at Oppenheimer citing the new licensing deal

Apr. 12, 2021 11:45 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | TSHA | By: Dulan Lokuwithana, SA News Editor

- Taysha Gene Therapies ([TSHA +16.1%](#)) has surged in the morning hours in response to its pre-market announcement of the acquisition of global rights to a clinical-stage [AAV9 gene therapy program](#).
- Expecting the deal to result in ‘important synergies,’ Oppenheimer which has an outperform rating on the stock has raised its price target by ~7.1% to \$45.00 per share indicating ~112.4% upside to the previous close.
- The acquisition that has consolidated the relationship with Steven Gray, chief scientific advisor Taysha Gene has sped up its commercialization prospects, the firm notes, calling the licensing terms ‘attractive.’
- In March, Taysha Gene announced a multi-year collaboration with Yong-Hui Jiang, a professor and chief of medical genetics at Yale University for AAV gene therapies [targeted at neurodevelopmental disorders](#).

Healthcare

Taysha Gene Therapies names Mary Newman as chief development officer

May 13, 2021 7:57 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) announces the appointment of industry veteran Mary Newman as the company's chief development officer.
- Newman joins Taysha with more than 30 years of experience in regulatory affairs and research and development within the biotechnology industry, focusing on rare diseases.
- Most recently, she served as Senior Vice President of Regulatory Affairs at Astellas Gene Therapies.

Healthcare

Biogen's risks fueling drug pricing debate Citi says, AstraZeneca remains top pick at BofA: in today's analyst action

Jun. 09, 2021 9:05 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock, CKPT Stock, AZN Stock, BIIB Stock** | FSTX | By: Dulan Lokuwithana, SA News Editor | 1 Comment



Photo by goir/iStock via Getty Images

Biogen's Aducanumab price tag could reignite debate on drug pricing: Citi

- With a \$56K/ year price tag for its newly-approved Alzheimer's disease treatment Aduhelm (aducanumab), Biogen (NASDAQ:[BIIB](#)) has already [generated criticism from lawmakers](#).
- The company is on track for the commercial launch of the therapy with "less preparation on the payer side," Citi analyst Mohit Bansal wrote with a neutral rating on the stock and the price target of \$440.00 per share indicating a premium of ~11.3%.

- Citing the total addressable market estimated at ~\$85 in the U.S. with more than 80% being on Medicare, “it creates an existential question for the program,” the analyst argues pointing to Medicare Part-B spending estimated at ~\$37B in 2019.
- As the debate on drug pricing appears to be winding down, “we suspect the industry could get right back into it again,” Bansal predicts cautioning potential pushback from payers.
- Biogen will begin shipping of Aduhelm in about two weeks, the company CEO [Michel Vounatsos said yesterday](#).

AstraZeneca remains the top pick at BofA

- Citing valuation, Bank of America keeps AstraZeneca (NASDAQ:[AZN](#)) as the firm’s top pick.
- The analysts Sachin Jain and the team argue that the stock trades at ~15x in terms of its pro forma 2022E EPS in line with the EU pharma sector despite the company’s better growth prospects and “significant pipeline optionality.”
- Assuming a ~22x PE multiple for 2022E, the analysts reiterate the buy rating with a price target of £103.00 (US\$69.40).
- AstraZeneca recently appointed Aradhana Sarin the former CFO of Alexion as the [company’s new Chief Financial Officer](#). In December, the company agreed to acquire Alexion [in a deal valued at \\$39B](#).

Checkpoint Therapeutics climbs after Bullish call from B Riley

- Checkpoint Therapeutics (NASDAQ:[CKPT](#)) has [surged ~33.5%](#) in the pre-market after B Riley analyst Justin Walsh initiated the coverage of the stock with a buy rating. The price target of \$18.00 per share implies a premium of ~576.7%.
- The analyst notes that Checkpoint’s lead asset Cosibelimab has been comparable to approved PD-(L)1 therapies and expects it to generate \$1.3B in peak net sales adjusted for risk as Walsh estimates more than \$15B sales in the U.S. for major approved PD-(L)1 inhibitors.

- The company is on track to release top-line results for Cosibelimab by year-end from a registration-enabling study in metastatic **cutaneous squamous cell carcinoma**.

F-star shines on B Riley initiation

- F-star Therapeutics (**FSTX**) has **added ~5.0%** in the pre-market after B Riley initiated the coverage of the stock with a buy rating and a 12-month price target of \$28.00 per share implying a premium of ~302.9%.
- The analyst Justin Walsh thinks that the clinical-stage company is well-positioned to establish itself in the competitive immuno-oncology field citing its lead asset FS118 which targets LAG-3, as well as PDL1.
- In H1 2022, F-star intends to give an update on the progress of a phase 2 proof-of-concept trial for FS118 in patients with PD-1/PD-L1 acquired **resistance head and neck cancer**.

Taysha Gene Therapies rates new buy at Wedbush

- Taysha Gene Therapies (NASDAQ:**TSHA**) has **gained ~1.4%** in the early hours after Wedbush initiated the coverage with an outperform rating. The price target of \$31.00 per share indicates a premium of ~30.3%.
- The firm points to the company's expansive pipeline with over 25 gene therapy candidates including TSHA-120 which according to the analyst has the potential to begin commercialization as early as 2023.
- The company expects data from the highest dose cohort in H2 2021 for TSHA-120 that is undergoing studies in patients with giant axonal neuropathy.

All share price moves are calculated based on the market close unless otherwise stated.

Healthcare

After recent weakness, gene therapy space offers a compelling opportunity: BTIG

Jun. 15, 2021 4:07 PM ET | **Sio Gene Therapies Inc. (SIOX) Stock, PASG Stock, DNTH Stock, AVRO Stock** | SRPT, QURE, AGTC... | By: Dulan Lokuwithana, SA News Editor | 6 Comments



dinn/iStock via Getty Images

- BTIG analysts Yun Zhong and Xu Zou witness “an attractive opportunity in gene therapy” after seeing that many players in the field are trading closer to “52-week low than to the 52-week-high.”
- The gene therapy candidates will see an “accelerated translation from the lab to the clinic,” thanks to improving knowledge on human biology and persistent innovation in technology, the firm noted.

- Following the recent FDA scrutiny on manufacturing and safety concerns, “we believe the recent weakness in the sub-sector is nothing but a maturing process,” the analysts argue as they initiate the coverage of several stocks with bullish ratings.
- Applied Genetic Technologies Corporation (NASDAQ:[AGTC](#)), AVROBIO (NASDAQ:[AVRO](#)), Magenta Therapeutics (NASDAQ:[MGTA](#)), Passage Bio (NASDAQ:[PASG](#)), Sarepta Therapeutics (NASDAQ:[SRPT](#)), Selecta Biosciences (NASDAQ:[SELB](#)), Sio Gene Therapies (NASDAQ:[SIOX](#)), Taysha Therapeutics (NASDAQ:[TSHA](#)), and uiQure (NASDAQ:[QURE](#)) have all received buy ratings from BTIG.
- Recently, RBC Capital Markets expanded its [coverage of gene-editing space](#).

Healthcare

Taysha initiated buy at Truist citing the platform and upcoming data reads

Jun. 24, 2021 4:42 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | TSHA | By: Dulan Lokuwithana, SA News Editor



naphtalina/iStock via Getty Images

- Truist initiated the coverage of Taysha Gene Therapies (NASDAQ:TSHA) with a buy recommendation predicting that positive updates in H2 2021 for two of the company's assets namely TSHA-120 and TSHA-101 "could serve as positive read-throughs" to Taysha's other pipeline programs.

- The analyst Joon Lee argues that the “vector, GMP manufacturing process and intrathecal route of delivery” used in Taysha’s other portfolio programs are similar to that used in TSHA-120 and TSHA-101.
- The 12-month price target at \$60.00 per share implies a premium of ~127.8% to the close. However, due to the nature of the company’s platform, the analyst forecasts ~2,000% upside and ~81.0% downside based on long-term bull/bear scenarios.
- In H2 2021 Taysha has two key data readouts. TSHA-120 undergoing studies for giant axonal neuropathy is on track to generate clinical data from a subset of patients while Phase 1/2 trial for TSHA-101 targeting GM2 gangliosidosis is set to report preliminary safety and biomarker data.

Healthcare

On the Move

Taysha Gene secures up to \$100M non-dilutive term loan financing

Aug. 16, 2021 8:27 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has entered into a loan and security agreement with Silicon Valley Bank (SVB) that provides Taysha with up to \$100M of borrowing capacity.
- TSHA shares [up 3.6% premarket](#) at \$18.
- This non-dilutive financing provides Taysha with up to \$100M, with \$40M available at closing of which Taysha has drawn \$30M.
- The Company has the option to draw down the remaining tranches. The interest rate is the greater of 7.0% or the WSJ Prime Rate plus 3.75%.
- There are no financial covenants and no warrants associated with the term loan.
- This funding will provide Taysha with operational and financial flexibility to achieve numerous value-generating milestones including a potential regulatory approval for TSHA-120 in giant axonal neuropathy.
- Earlier today, the company reported financial results for [Q2 ended June 30, 2021](#).

Healthcare

Taysha Gene's TSHA-105 nabs Orphan Drug tag in Europe

Aug. 25, 2021 7:25 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | CNS | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has been granted orphan drug designation from the European Commission for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy, a form of infantile epilepsy.
- Among the benefits of Orphan Drug status in Europe is a 10-year period of market exclusivity for the indication, protocol assistance, reduced regulatory, if approved.
- TSHA-105 is first program in Taysha's pipeline to receive designation from European Commission

Healthcare

Taysha Gene's TSHA-102 an Orphan Drug in Europe for Rett Syndrome

Sep. 22, 2021 7:40 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team | 1 Comment



designer491/iStock via Getty Images

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has been granted orphan drug designation (ODD) from the European Commission for TSHA-102, an AAV9-based gene replacement therapy in development for Rett syndrome.
- Among the benefits of Orphan Drug status in Europe is a 10-year period of market exclusivity for the indication, protocol assistance, reduced regulatory, if approved.

- Rett syndrome is a severe genetic neurodevelopmental disorder characterized by rapid developmental regression that leads to intellectual disabilities, loss of speech, loss of mobility, seizures, cardiac impairments and breathing issues. Currently, there are no approved therapies.
- Last month, [Taysha won Orphan Drug tag in Europe for TSHA-105](#).

Healthcare

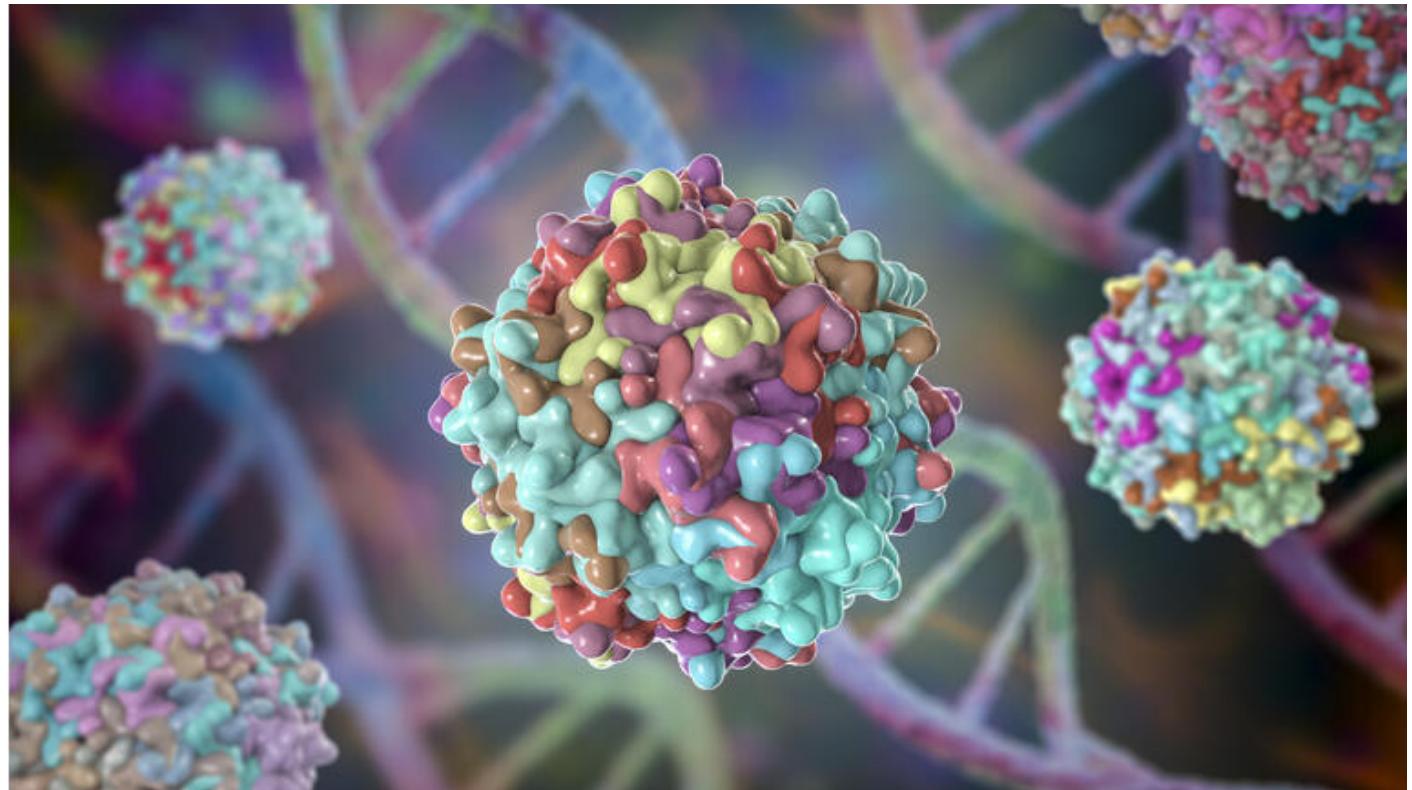
Taysha Gene's TSHA-101 an Orphan Drug in Europe for infantile GM2 gangliosidosis

Sep. 29, 2021 7:19 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has been granted orphan drug designation from the European Commission for TSHA-101, an AAV9-based bicistronic gene replacement therapy in development for GM2 gangliosidosis, also called Tay-Sachs or Sandhoff disease.
- GM2 gangliosidosis is a fatal neurodegenerative disease caused by deficiency in the lysosomal enzyme β -hexosaminidase A, also known as Hex A.
- Among the benefits of Orphan Drug status in Europe is a 10-year period of market exclusivity for the indication, protocol assistance, reduced regulatory, if approved.
- TSHA-101 is the first and only bicistronic vector currently in clinical development and has been granted Orphan Drug and Rare Pediatric Disease designations by the FDA.
- Recently, TSHA-102 won [Orphan Drug tag in Europe for Rett Syndrome](#).

Taysha licenses worldwide rights to AAV9 gene therapy program for CLN7 disease

Oct. 05, 2021 7:23 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team



Dr_Microbe/iStock via Getty Images

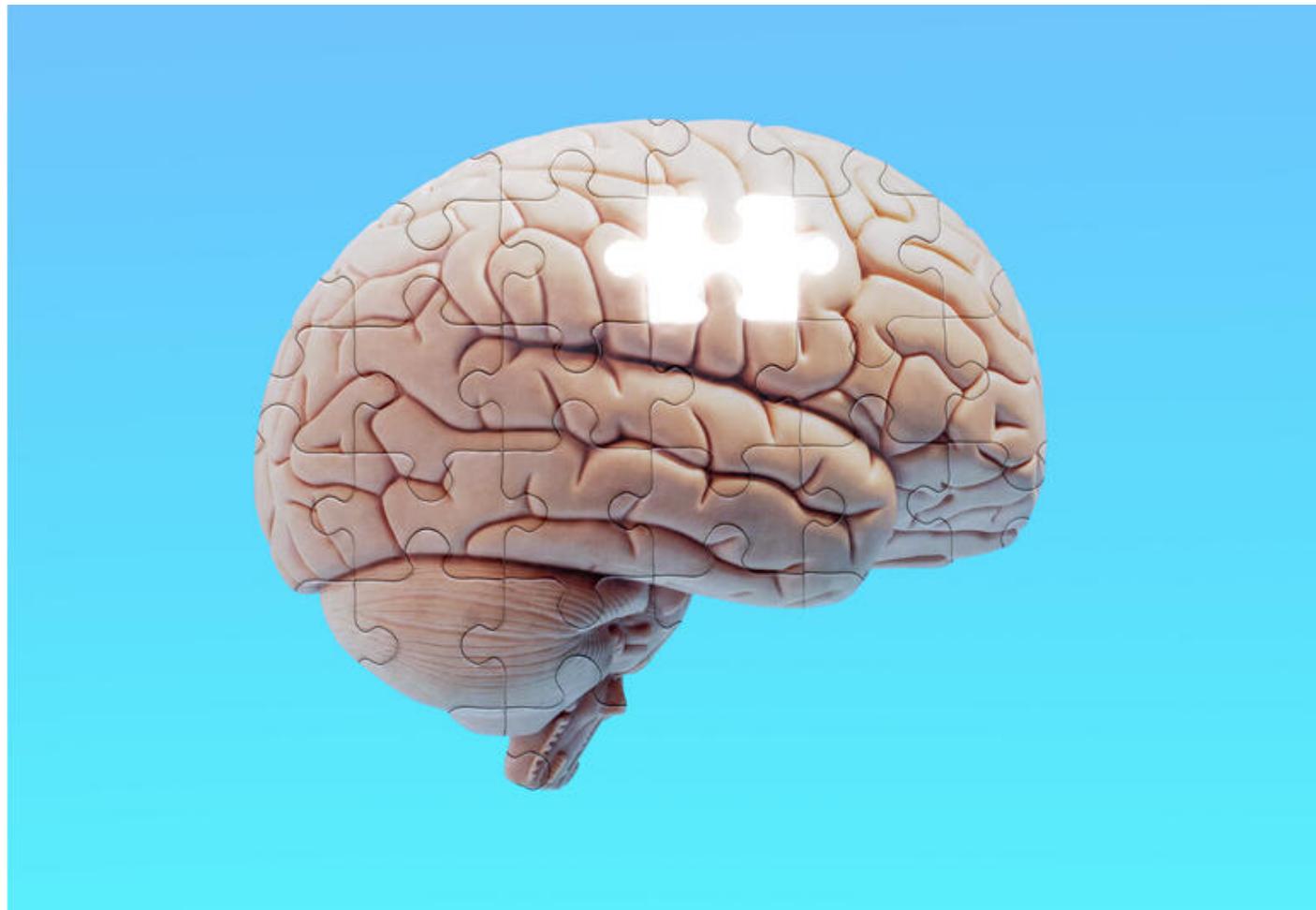
- Taysha Gene Therapies (NASDAQ:[TSHA](#)) has obtained an exclusive option from UT Southwestern (UTSW) to license worldwide rights to a clinical-stage AAV9 gene therapy replacement program for the treatment of CLN7 disease.
- The company has also entered into a research collaboration with UTSW to develop a next-generation construct for the treatment of CLN7 disease.
- Financial terms of the agreements were not disclosed.
- Completion of the next-generation construct design is anticipated by year-end 2021, with commercial-grade GMP material expected in 2022.

- The CLN7 program is currently in a Phase 1 clinical proof-of-concept trial, and Taysha expects preliminary safety and efficacy data from first-generation construct by year-end. TSHA intends to initiate a planned pivotal trial using a next-generation construct in 2022.
- CLN7 disease is a rare, fatal and rapidly progressive neurodegenerative disease that is a form of Batten disease. Patients experience gradual nerve cell loss in certain parts of the brain and typically present with seizures, vision loss, speech impairment and mental and motor regression.

Healthcare

Taysha Gene Therapies inks genetic testing partnership with GeneDx

Oct. 13, 2021 7:44 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team



PM Images/DigitalVision via Getty Images

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) announces sponsored genetic testing for giant axonal neuropathy (GAN) in partnership with GeneDx.
- GAN is a progressive neurodegenerative disease that affects both the central and peripheral nervous systems leading to motor weakness, sensory impairment, and cognitive dysfunction.

- Under the partnership, Taysha will sponsor the inclusion of a genetic marker to test for GAN in the GeneDx hereditary neuropathy panel free of charge to individuals at risk for or suspected of having GAN.
- GeneDx will provide analysis of genes on the Hereditary Neuropathy Panel by next-generation sequencing with deletion/duplication detection.

Healthcare

Taysha Gene Therapies highlights proof-of-concept data for Angelman Syndrome treatment

Oct. 25, 2021 7:32 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: SA News Team



PM Images/DigitalVision via Getty Images

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) highlights the publication of positive proof-of-concept preclinical data for an AAV-mediated UBE3A gene replacement approach demonstrating therapeutic potential for the treatment of Angelman Syndrome (AS).

- Anatomical and behavioral phenotypes were recovered following treatment, providing proof-of-concept preclinical data supporting further study of UBE3A gene replacement therapy as a potentially safe and effective treatment for AS, the company said.
- AS is a monogenic neurodevelopmental disorder caused by deletions or mutations in the maternal ubiquitin protein ligase E3A (UBE3A) gene.
- There are currently no approved treatments for AS and current interventions are focused on managing medical and developmental issues.

Healthcare

Taysha shares pre-clinical data for gene therapy in epilepsy due to SLCA13A5 deficiency

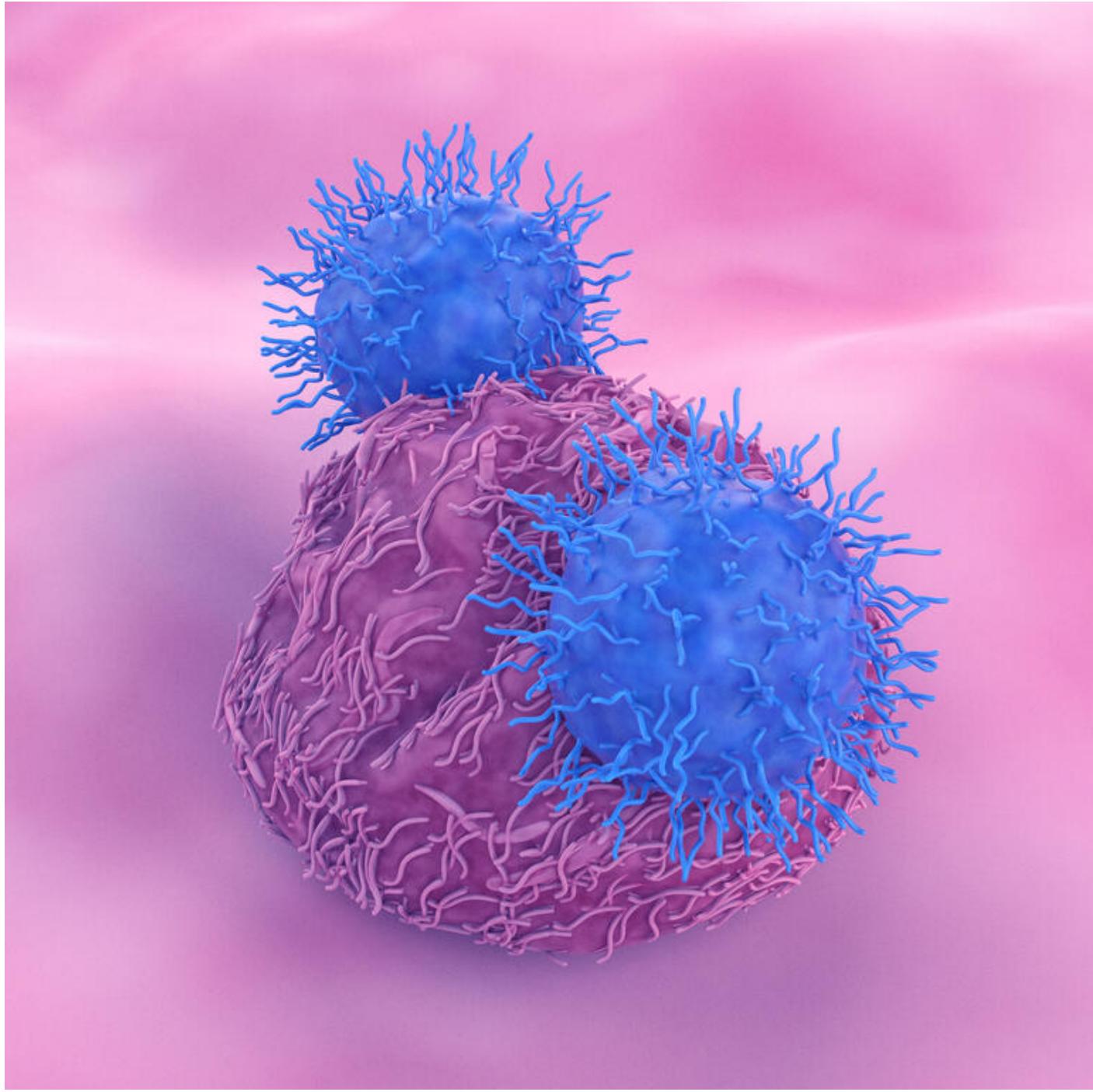
Dec. 06, 2021 4:47 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

- Disclosing a late-breaking abstract and poster presentation conducted at a medical event, Taysha Gene Therapies (NASDAQ:[TSHA](#)) says it expects to seek regulatory clearances to start clinical trials for its experimental gene therapy TSHA-105 in patients with SLC13A5-related epilepsy.
- SLC13A5-related epilepsy is characterized by a mutation that prevents brain cells from taking up citrate. However, according to pre-clinical data presented at the American Epilepsy Society Annual Meeting on Monday, TSHA-105 has led to normalized citrate levels in mouse studies.
- In knockout mouse models with the deficiency, there was a decline in seizure activity and improvement in the survival regardless of age, Rachel M. Bailey, Assistant Professor with the Center for Alzheimer's and Neurodegenerative Diseases and Pediatrics at UT Southwestern said. "We are highly encouraged by the positive therapeutic response and absence of toxicity in these preclinical models."
- "These highly encouraging preclinical data further support our plan to submit an IND/CTA filing in 2022," remarked Suyash Prasad, Taysha's ([TSHA](#)) Chief Medical Officer. The company shares have **added ~10.1%** in the post-market.
- Read more on upcoming milestones for the biotech, as announced by its management last month.

[Healthcare](#)[On the Move](#)

Taysha Gene Therapies rises 4% following Guggenheim buy rating; sees 97% upside

Dec. 16, 2021 1:37 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Jonathan Block, SA News Editor
| 1 Comment



Iuismmolina/iStock via Getty Images

- Taysha Gene Therapies (**TSHA +4.6%**) is up today after Guggenheim initiated what it calls a "pure-play" central nervous system gene therapy company.
- The firm has a \$28 price target (~97% upside based on yesterday's close).
- Analyst Debjit Chatopadyaya says that additional regulatory clarity and clinical updates from up to four programs by the end of next year could bolster its platform.

- Taysha's candidates use an adeno-associated virus 9 (AAV9) capsid to deliver therapeutics genes directly into the body's cerebrospinal fluid.
- The company's lead candidate, TSHA-120, is a gene replacement therapy in phase 1/2 for giant axonal neuropathy.
- Chattopadyaya says the company's \$400M enterprise value provides an attractive entry point, as well as the **46% decline** in shares year to date.
- Read about recent [pre-clinical data for TSHA-105 in epilepsy](#).

Healthcare

Taysha shares early safety data for first-generation construct in rare neuro disease

Dec. 22, 2021 7:27 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

- Announcing the preliminary clinical safety data for the company's first-generation construct in CLN7 disease, Taysha Gene Therapies (NASDAQ:[TSHA](#)) said that the experimental therapy demonstrated a favorable tolerability and safety profile with no major adverse events across the doses.
- CLN7 disease is a rare and fatal neurodegenerative disease and a form of Batten disease. So far, three patients have received the therapy, including two of them at a 1.0×10^{15} total vg dose, the highest ever dose for a gene therapy administered intrathecally in humans, according to the company.
- The data will be presented at the 18th Annual WORLDSymposium in February 2022 by Dr. Ben Greenberg, a professor in the departments of Neurology ad Pediatrics at UT Southwestern (UTSW).
- "UTSW has finalized the design of a next-generation CLN7 construct, which is expected to improve potency, packaging efficiency and manufacturability as well as reduce risk of immunogenicity over the first-generation construct," Suyash Prasad, Chief Medical Officer of Taysha ([TSHA](#)) noted. The next-generation construct is set to reach a pivotal trial in 2022, he added.
- Read more on the upcoming milestones for the company, as announced by the management in November.

Healthcare

Taysha posts early biomarker data for gene therapy in GM2 gangliosidosis

Jan. 27, 2022 7:48 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) announced encouraging initial biomarker data for its experimental gene therapy TSHA-101 in patients with Sandhoff and Tay-Sachs diseases, two forms of GM2 gangliosidosis.
- The Hex A enzyme levels in patients with asymptomatic GM2 gangliosidosis – a genetically inherited disorder of the brain and spinal cord – stand at least 5% of normal activity, based on natural history data.
- However, following the treatment with TSHA-101, patient 1 with Sandhoff disease indicated 38-fold and 58-fold of Hex A enzyme levels above the asymptomatic level at month 1 and month 3, respectively. Patient 2 with Tay-Sachs disease showed a 5-fold above the asymptomatic level at month 1.
- According to initial findings, TSHA-101 was well tolerated with no significant drug-related events.
- “We look forward to submitting a protocol amendment to expand patient enrollment in the ongoing Phase 1/2 trial and providing additional updates later this year,” Chief Medical Officer of Taysha Gene Therapies, Suyash Prasad, said.
- In December, the company announced that the European Commission granted the orphan drug [designation for TSHA-101](#).

Healthcare

Taysha posts data from high dose cohort for TSHA-120 in giant axonal neuropathy

Jan. 31, 2022 8:49 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor

Announcing the data for high dose cohort of TSHA-120 in rare inherited disease, giant axonal neuropathy, Taysha Gene Therapies (NASDAQ:[TSHA](#)) noted that the experimental therapy led to clinically meaningful and statistically significant improvement in patient outcomes.

The high dose cohort of 3.5×10^4 total vg led to a 5-point improvement in the change in the rate of decline in MFM32 score by year 1, compared to natural history decline of 8 points, the company said. 32-item Motor Function Measure (MFM32) is a key measure used to assess the functional abilities of patients with neuromuscular diseases.

Across all therapeutic dose cohorts, there was a 7-point improvement in the rate of decline in MFM32 score by year 1, compared to natural history decline of 8 points.

By year 3, the mean change in MFM32 was a 10-point improvement for all therapeutic dose cohorts, compared to the estimated natural history decline of 24 points.

RA Session II, CEO of Taysha ([TSHA](#)), says that the data could support the future regulatory path for TSHA-120 as the company finalizes its commercial strategy targeting an estimated 5,000 patients in the addressable markets.

“The totality of data generated by TSHA-120 to date support our plans to engage with major regulatory agencies in order to discuss pathways for registration, and we look forward to providing a regulatory update later this year,” he said.

In contrast with the Strong Buy recommendation for Taysha ([TSHA](#)) on Wall Street, Seeking Alpha Quant rating indicates a Strong Sell [recommendation for the stock](#).

[Tech](#) [Energy](#) [Consumer](#) [Cryptocurrency](#)

Catalyst watch: Apple earnings, Twitter drama and media eyes on NAB Show

Apr. 22, 2022 3:00 PM ET | **Apple Inc. (AAPL) Stock, TWTR Stock, SEVCQ Stock, NFLX Stock** | IMAX, AUDA, BYON... | By: Clark Schultz, SA News Editor | 15 Comments



shapecharge/iStock via Getty Images

Welcome to Seeking Alpha's Catalyst Watch - a breakdown of some of next week's actionable events that stand out. Check out Saturday morning's regular [Stocks to Watch article](#) for a full list of events planned for the week or the [Seeking Alpha earnings calendar](#) for companies due to report.

Monday - April 25

- **Volatility watch** - Options trading is elevated on Aytu BioPharma ([AYTU](#)) and Sonos Group N.V. (NASDAQ:[SEV](#)). No surprise, but Netflix (NASDAQ:[NFLX](#)) and

Twitter (NYSE:[TWTR](#)) are right at the top of the list of most discussed stocks on Reddit's WallStreetBets and StockTwits. Short interest as a percentage of total float is elevated again on Skillz ([SKLZ](#)) and Cinemark Holdings ([CNK](#)).

- **All day** - The NAB Show runs through April 27. The world's largest annual conference for broadcast, entertainment and technology professionals will feature exhibiting companies from 38 nations. Sinclair Broadcast Group ([SBGI](#)), Veritone ([VERI](#)), and Quantum Corporation ([QMCO](#)) have highlighted their appearances this year. Some of the key stocks to watch for potential analyst reactions are Gray Television ([GTN](#)), Nexstar Media Group ([NXST](#)), E.W. Scripps Company ([SSP](#)), TEGNA ([TGNA](#)), Audacy ([AUD](#)), and iHeartMedia ([IHRT](#)).

Tuesday - April 26

- **All day** - The Surface Transportation Board will hold public hearings to address recent service issues and recovery efforts. Executives from Burlington Northern Santa Fe, CSX Corporation ([CSX](#)), Norfolk Southern ([NSC](#)), and Union Pacific ([UNP](#)), Canadian National ([CN](#)), Canadian Pacific ([CP](#)), and Kansas City Southern are expected to attend the hearing. The focus of the hearings will be to push the rails companies to detail action plans to address the issues. Ahead of the hearing, Union Pacific said congestion on tracks was hurting its ability to meet shipping demand.
- **Premarket** - The Chardan 6th Annual Genetic Medicines Manufacturing Summit 2022 will include presentations from REGENXBIO ([RGNX](#)), Wave Life Sciences Ltd. ([WVE](#)), Century Therapeutics ([IPSC](#)), Achilles Therapeutics plc ([ACHL](#)), Taysha Gene Therapies ([TSHA](#)), Passage Bio ([PASG](#)) and Tenaya Therapeutics ([TNYA](#)). The event has led to share price spikes for participating companies in the past.
- **All day** - Shareholders with Archimedes Tech SPAC Partners Co. ([ATSPT](#)) will vote on the deal to take AI firm SoundHound public. The company has added \$2 million to the PIPE to bring the deal's total PIPE proceeds to \$113 million.
- **All day** - Bull Horn Holdings Corp. ([BHSE](#)) holds a meeting to vote on extending the deadline to combine with Coeptis Therapeutics ([OTCPK:COEP](#)) in a SPAC deal.

- **All day** - Shareholders with 51job ([JOBS](#)) formally vote on the deal to take a buyout offer from DCP Capital and Ocean Link Partners.
- **10:15 a.m.** Appian ([APPN](#)) holds an investor session at its Appian World 2022 event. Shares of Appian have rallied after similar events.
- **11:00 a.m.** Callaway Golf Company ([ELY](#)) will hold an Investor Day event with presentations and Q&A sessions led by members of the Callaway and Topgolf executive leadership teams.
- **Postmarket** - Companies reporting earnings with double-digit share price moves up or down implied by options trading include QuantumScape ([QS](#)), Sigma Labs ([SGLB](#)), Teradyne ([TER](#)) and Boyd Gaming ([BYD](#)).
- **Postmarket** - Nasdaq is due to post its short interest report.

Wednesday - April 27

- **All day** - ACM Research ([ACMR](#)) will release its preliminary revenue range for Q1 ahead of the full earnings report due in on May 6.
- **All day** - Companies reporting earnings with double-digit share price moves up or down implied by options trading include Spotify ([SPOT](#)) and Steven Madden ([SHOO](#)).
- **All day** - Praxis Precision Medicines ([PRAX](#)) will host an Epilepsy Day event. During the event, Praxis' management team will review the company's Epilepsy franchise.
- **All day** - Crypto will see more global exposure with the first spot Bitcoin ([BTC-USD](#)) and spot Ethereum ([ETH-USD](#)) exchange traded funds launching in Australia on the CBOE Exchange.
- **12:00 p.m.** Vir Biotechnology ([VIR](#)) will host a virtual Hepatitis Portfolio R&D Day for the investment community.
- **4:30 p.m.** Frontier Group Holdings ([ULCC](#)) holds its earnings call following the airline company's Q1 earnings report. The call will be closely watched within the

airline sector with both Frontier and JetBlue Airways ([JBLU](#)) looking to acquire Spirit Airlines ([SAVE](#)).

Thursday - April 28

- **All day** - Spring Valley Acquisition Corp. ([SV](#)) will hold a shareholder meeting to vote on taking NuScale Power public in a SPAC deal. NuScale Power says its mission is to provide scalable advanced nuclear technology for the production of electricity, heat, and clean water to improve the quality of life for people around the world. The SPAC deal will value the combined company at about \$1.9B. Fluor ([FLR](#)) and Nucor Corporation ([NUE](#)) are also investors in NuScale Power.
- **All day** - Activision Blizzard ([ATVI](#)) shareholders will vote on Microsoft's ([MSFT](#)) buyout offer.
- **Premarket** - Companies reporting earnings with double-digit share price moves up or down implied by options trading include Limelight Networks ([LLNW](#)), United States Steel ([X](#)), Overstock.com ([OSTK](#)) and Peabody Energy ([BTU](#)).
- **8:00 a.m.** Twitter ([TWTR](#)) will hold its earnings call after reporting earnings. A key question around the efforts of Elon Musk to buy the company is if a second bidder emerges, which could force Musk to increase his offering price.
- **11:00 a.m.** Eledon Pharmaceuticals ([ELDN](#)) will hold a virtual R&D Day event. The presentations will include a deep dive on the company's investigational drug, tegoprubart, and the promise of CD40 Ligand targeted immunotherapy for the treatment of amyotrophic lateral sclerosis, kidney transplantation, islet cell transplantation, and IgA nephropathy. Shares of Eledon Pharmaceuticals are down 33% YTD.
- **11:00 a.m.** Blue Apron ([APRN](#)) will participate in the Jefferies Online Meal Kit Summit to discuss the next avenues for growth and the possible effects of inflation on the meal kit sector. There has been volatile trading on Blue Apron ([APRN](#)) over the last few months with the broad online retail sector.
- **Postmarket** - Companies reporting earnings with double-digit share price moves up or down implied by options trading include Robinhood Markets ([HOOD](#)), Roku ([ROKU](#)), and IMAX ([IMAX](#)).

- **5:00 p.m.** Apple (NASDAQ:[AAPL](#)) holds its earnings call during which some analysts think the tech giant could provide new annual capital return guidance on buybacks and the dividend payout. Since announcing its initial share buyback and dividend strategy in March of 2021, Apple has cumulatively returned more than 95% of its free cash flow to investors. A wildcard during the call would be a mention of any progress with the iCar. A recent patent filing from Apple claimed protection for a "vehicle that has a plurality of cameras, a network interface, a memory, and one or more computer processors."

Friday - April 29

- **Premarket** - Earnings from the energy sector will come in from Exxon Mobil ([XOM](#)), Chevron ([CVX](#)) and Phillips 66 ([PSX](#)). Those reports could spill over to impact shares prices on dozens of companies. ProPetro Holding ([PUMP](#)) has traded in the same direction as Exxon on earnings days eight consecutive times, while Talos Energy ([TALO](#)) has followed Chevron directionally eight straight earnings days as well.
- **All day** - Deutsche Bank ([DB](#)) will hold a fixed income investor conference call to go over Q1 results.
- **All day** - Trupanion ([TRUP](#)) will hold its annual Q&A session in Omaha, Nebraska.

[Healthcare](#)[On the Move](#)

Bluebird Bio drives gene therapy stocks higher after FDA review

Jun. 07, 2022 3:59 PM ET | **bluebird bio, Inc. (BLUE) Stock** | NVS, SGMO, NVSEF... | By: Dulan Lokuwithana, SA News Editor | 12 Comments



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- A group of biotechs focused on gene therapy candidates surged on Tuesday after the FDA posted a favorable review on a β-thalassemia candidate developed by commercial-stage company Bluebird bio (NASDAQ:[BLUE](#)).
- The data for the marketing application of one-time gene therapy beti-cel “support the effectiveness” of the drug for the targeted indication with a “clinically

meaningful benefit," the FDA reviewers wrote.

- Despite safety concerns over its second gene therapy candidate eli-cel for cerebral adrenoleukodystrophy, Bluebird bio ([BLUE](#)) has posted its best intraday gain since 2014.
- Other notable gainers in the gene therapy space include Homology Medicines ([FIXX](#)), Selecta Biosciences ([SELB](#)), Sio Gene Therapies ([SIOX](#)), Taysha Gene Therapies ([TSHA](#)), Iovance Biotherapeutics ([IOVA](#)), uniQure N.V. ([QURE](#)), and Voyager Therapeutics ([VYGR](#)).
- Gene-editing companies such as Precision BioSciences ([DTIL](#)), Sangamo Therapeutics ([SGMO](#)), Verve Therapeutics ([VERV](#)), Editas Medicine ([EDIT](#)), and Beam Therapeutics ([BEAM](#)) have also recorded modest gains.
- Novartis ([NVS](#)) ([OTCPK:NVSEF](#)) company AveXis last won the FDA approval for its gene therapy candidate Zolgensma for children with [spinal muscular atrophy](#) in [2019](#).

Earnings News

Taysha Gene Therapies GAAP EPS of -\$0.84 beats by \$0.13

Aug. 11, 2022 7:17 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Pranav Ghumatkar, SA News Editor

- Taysha Gene Therapies [press release](#) (NASDAQ:[TSHA](#)): Q2 GAAP EPS of -\$0.84 beats by \$0.13.
- As of June 30, 2022, the Company had cash and cash equivalents of \$66.2 million.
- Taysha continues to expect that its current cash and cash equivalents, in addition to full access to its existing term loan facility, is sufficient to fund operating expenses into the fourth quarter of 2023.

[Healthcare](#)[On the Move](#)[M&A](#)

Taysha Gene stock soars 36% as Astellas to pick 15% stake for \$50M

Oct. 25, 2022 6:29 AM ET | **Astellas Pharma Inc. (ALPMY) Stock, TSHA Stock, ALPMF Stock** | By: Ravikash, SA News Editor | 1 Comment



ktsimage

Japan's Astellas Pharma ([OTCPK:ALPMF](#)) ([OTCPK:ALPMY](#)) is acquiring a 15% stake in Taysha Gene Therapies ([NASDAQ:TSHA](#)) for \$50M to support the advancement of Taysha's gene therapy programs for Rett syndrome and giant axonal neuropathy (GAN).

Under the agreement, Astellas will invest \$50M to acquire 15% of the outstanding common stock of Taysha and to receive an exclusive option to license two clinical stage programs.

The first is TSHA-102 for Rett syndrome — rare genetic neurological and developmental disorder affecting brain development and causes loss of movement functions. The disorder affects mostly girls.

The second is TSHA-120 for GAN — a rare inherited genetic disorder which affects the central and peripheral nervous systems.

Astellas will also receive one board observer seat at Taysha.

In addition, Taysha said it granted Astellas certain rights related to any potential change of control of the company.

Definitive agreements would be executed if Astellas exercises any such option, and any change of control transaction would need approval by Taysha's stockholders, the company added.

"Taysha is an industry leader in CNS gene therapies and this partnership fits strategically with our long-term vision of expanding Astellas' gene therapy capabilities," said Astellas Chief Strategy Officer Naoki Okamura.

TSHA +36.42% to \$2.06 premarket Oct. 25

[Healthcare](#)[On the Move](#)

Taysha Gene Therapies announces \$30M stock offering; shares fall 14% after hours

Oct. 25, 2022 4:07 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Jessica Kuruthukulangara, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) said Tuesday it intends to offer and sell \$30M of shares in an underwritten public offering.
- Shares of Taysha Gene ([TSHA](#)), which ended **97.4% higher**, **fell 14.1%** after hours.
- The underwriter will get a 30-day option to buy up to an additional 15% of the shares in the offering.
- There can be no assurance as to whether or when the offering may be completed.

[Healthcare](#)[On the Move](#)

Gene therapy firm Taysha prices \$28M stock offering

Oct. 27, 2022 8:50 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Preeti Singh, SA News Editor

- Taysha Gene Therapies (NASDAQ:TSHA) shares fell **16%** premarket on Thursday after the gene therapy company [priced](#) its \$28M stock offering.
- The offering comprises 14M shares of its common stock issued at \$2/share.
- In addition, Taysha (TSHA) has granted the underwriter a 30-day option to purchase up to 2.1M additional shares of common stock at the public offering price, less the underwriting discount.
- Gross proceeds are expected to be approximately \$28M.
- The offering is expected to close on or about October 31, 2022.

Healthcare

Taysha Gene Therapies downgraded to neutral at Goldman Sachs on measured outlook

Nov. 09, 2022 11:55 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | ALPMF, ALPMY | By: Jonathan Block, SA News Editor | 4 Comments



Fabrice Cabaud

- Goldman Sachs has downgraded Taysha Gene Therapies (NASDAQ:TSHA) to neutral from buy saying it has a more measured outlook on its portfolio.
- The firm also cut its price target to \$3 from \$16 (~36% upside based on Tuesday's close).
- Analyst Salveen Richter said that while she is encouraged by the recent strategic investment by Astellas Pharma (OTCPK:ALPMF)(OTCPK:ALPMY), she is hesitant

on the stock due to the company's reprioritization of assets.

- She is removing gene therapy programs for SLC6A1, SURF1, CLN1 from her model.
- Seeking Alpha's Quant Rating [views Taysha \(TSHA\)](#) as a strong sell.

Healthcare

Taysha Gene Therapies names new CEO

Dec. 19, 2022 3:03 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Manshi Mamtora, CFA

- Taysha Gene Therapies' (NASDAQ:TSHA) Chair of the Board of Directors, Sean P. Nolan, a highly experienced biopharmaceutical industry senior leader, [has been appointed](#) CEO, succeeding RA Session II, who has resigned from his operating role, but will continue to serve on the Company's Board of Directors.
- In addition, Sukumar Nagendran, M.D., a Director on Board of Directors, and an accomplished physician, drug developer, and biotech executive, has been appointed President and Head of R&D.
- Mr. Nolan previously served as CEO of the gene therapy company, AveXis, until its acquisition by Novartis.

Healthcare

On the Move

Taysha falls as FDA seeks additional dosing for CNS disorder candidate

Jan. 31, 2023 4:29 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | ALPMF, ALPMY | By: Dulan Lokuwithana, SA News Editor



hapabapa

Taysha Gene Therapies (NASDAQ:[TSHA](#)) fell ~6% post-market Tuesday after announcing that the FDA recommended dosing more patients to support a marketing application for TSHA-120 in rare central nervous system disorder giant axonal neuropathy (GAN).

The company said that the FDA suggests a double-blind, placebo-controlled design for additional dosing. The decision follows a Type B end-of-Phase 2 meeting Taysha ([TSHA](#)) conducted with the FDA regarding TSHA-120, for which Japan-based Astellas Pharma ([OTCPK:ALPMF](#)) ([OTCPK:ALPMY](#)) has partnered.

The company is waiting for the FDA's response regarding its follow-up questions on the study design and overall data required for a marketing application.

Additionally, the company announced plans to dose the first adult patient for its Rett syndrome candidate TSHA-102 in H1 2023.

Taysha ([TSHA](#)) also said that a comprehensive strategic review implemented to improve business execution has led to operational, structural, and personnel changes.

More details on FDA feedback and the corporate update are expected at a conference call scheduled for [Tuesday at 4:30 pm ET](#).

With a licensing deal for GAN and Rett syndrome programs, Astellas ([OTCPK:ALPMF](#)) "threw Taysha a \$50M lifeline," Seeking Alpha contributor Jonathan [Faison](#) argued in [November](#).

Healthcare

On the Move

Taysha downgraded at Jefferies citing uncertain outlook for lead asset

Feb. 01, 2023 1:00 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | ALPMF, ALPMY | By: Dulan Lokuwithana, SA News Editor



naphtalina/iStock via Getty Images

Taysha Gene Therapies (NASDAQ:[TSHA](#)) hit a 52-week low on Wednesday as Jefferies downgraded the biotech on the news that the FDA recommended additional dosing to support a marketing application for its lead gene [therapy candidate TSHA-120](#).

Taysha ([TSHA](#)) has partnered with Japan-based Astellas Pharma ([OTCPK:ALPMF](#)) ([OTCPK:ALPMY](#)) to develop TSHA-120 for the rare central nervous system disorder giant axonal neuropathy (GAN).

As the FDA has suggested a double-blind, placebo-controlled design for additional dosing, Jefferies analyst Eun K. Yang argued that the "regulatory path & future of TSHA-120 seem uncertain."

Yang downgrades TSHA to Hold from Buy and slashes its price target to \$1.50 from \$14.00, noting that the regulator has recommended a new trial despite the management's previous plans to launch TSHA-120 by the year-end or early 2023.

The analyst also sees slow progress in Taysha's ([TSHA](#)) second asset TSHA-102 and argues the company's limited cash resources, estimated at \$90M, can fund its operations only into Q1 2024.

Wall Street has remained bullish on Taysha ([TSHA](#)) stock, with an average rating of [Strong Buy from analysts](#) in line with a [Buy rating from Seeking Alpha Authors](#). However, Seeking Alpha's Quant System, [which consistently beats the market, rated TSHA as a Hold](#).

Healthcare

FDA to back accelerated approval pathway for gene therapies

Mar. 21, 2023 7:43 AM ET | **Sarepta Therapeutics, Inc. (SRPT) Stock** | BMRN, IOVA, BLUE... | By: Dulan Lokuwithana, SA News Editor | 6 Comments



Grandbrothers/iStock Editorial via Getty Images

A top FDA official said Monday that the agency needs to consider accelerated approval, a regulatory avenue commonly used to expedite the market entry of cancer drugs, for gene therapies.

Dr. Peter Marks, who heads the FDA's biologics unit, said that the agency will support the use of measurable biomarkers in gene therapy clinical studies as substitutes for other biological indicators to help developers secure "accelerated approval."

The FDA's accelerated approval program focuses on surrogate endpoints such as radiographic images and physical signs that are believed to predict clinical benefit but are not direct measures of a clinical benefit.

The program accelerates the market entry of drugs for unmet medical needs as the use of surrogate endpoints cuts the approval time.

"The FDA views gene therapy as an [excellent opportunity](#) to expedite the delivery of potentially life-saving therapies to patients with rare diseases," Dr. Marks, the director of FDA's Center for Biologics Evaluation and Research, said.

His comments came days after Sarepta Therapeutics (NASDAQ:[SRPT](#)), which seeks accelerated approval for its gene therapy SRP-9001 in Duchenne muscular dystrophy, said that the FDA plans to hold an AdCom meeting to discuss the [approvability of the treatment](#).

Other leading gene therapy developers: bluebird bio ([BLUE](#)), CRISPR Therapeutics ([CRSP](#)), Iovance Biotherapeutics ([IOVA](#)), uniQure ([QURE](#)), Taysha Gene Therapies ([TSHA](#)), BioMarin Pharmaceutical ([BMRN](#)), REGENXBIO ([RGNX](#))

A potential market entry of SRP-9001 will help accelerate Sarepta's ([SRPT](#)) "strong" growth trends, Seeking Alpha [contributor Biologics argued recently](#).

Earnings News

On the Move

Taysha Gene Therapies GAAP EPS of -\$0.99 misses by \$0.70, revenue of \$2.5M misses by \$6.5M

Mar. 28, 2023 4:21 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Pranav Ghumatkar, SA News Editor

- Taysha Gene Therapies [press release](#) (NASDAQ:[TSHA](#)): Q4 GAAP EPS of -\$0.99 **misses by \$0.70**.
- Revenue of \$2.5M **misses by \$6.5M**.
- As of December 31, 2022, Taysha had \$87.9 million in cash and cash equivalents. The Company continues to expect that its current cash resources will support planned operating expenses and capital requirements into the first quarter of 2024.
- Shares **+4.68%**.

Healthcare

Taysha Gene Therapies announces offering by selling stockholders - filing

Apr. 05, 2023 5:14 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Ahmed Farhath, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) on Wednesday said its selling stockholders intend to offer and sell 8.5 million shares of the company through a secondary offering.
- The company will not receive any proceeds from the offering.
- [SEC Filing](#).

Healthcare

Taysha Gene Therapies agrees on ~\$150M private placement financing

Aug. 14, 2023 8:26 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Mary Christine Joy, SA News Editor



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- Gene therapy company Taysha Gene Therapies (NASDAQ: [TSHA](#)) said on Monday that it has entered into a securities purchase agreement for an ~\$150M private placement financing.
- The financing is expected involve a sale of an aggregate of 122.41M shares at \$0.90 per share, and pre-funded warrants to purchase up to an aggregate of

44.25M shares at \$0.899 per pre-funded warrant.

- The financing was led by a new investor, RA Capital Management.
- CEO Sean Nolan said that the net proceeds are expected to be used to extend cash runway into Q3, working capital and other general corporate purposes.
- The financing was priced to satisfy Nasdaq's minimum price requirement.
- The PIPE is expected to close by Aug. 16.
- TSHA shares were trading **+67.79%** pre-market.
- Source: [Press Release](#)

Healthcare

On the Move

Taysha surges on private placement, early data from lead program

Aug. 14, 2023 11:56 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor



designer491

Taysha Gene Therapies (NASDAQ:[TSHA](#)) added ~126% in value after announcing a \$150M private placement and early data from its lead program REVEAL Phase 1/2 trial for gene therapy, TSHA-102, for the rare [neurodevelopmental disorder Rett syndrome](#).

Citing results from REVEAL's low-dose cohort, Taysha ([TSHA](#)) said that the first adult patient to receive TSHA-102 tolerated the investigational therapy well, indicating no treatment-emergent serious adverse events over six weeks.

Additionally, four weeks after the treatment, the patient demonstrated improvements across key efficacy measures, including the Rett Syndrome Behavior Questionnaire (RSBQ).

The company intends to dose the second patient in Q3 2023 and has received FDA clearance to expand the investigations into pediatric patients with Rett syndrome.

The scope of the efficacy shown was "unexpected" as the patient had only received a low dose, analyst Kristen Kluska argued, after the results, raising her price target on TSHA to \$7 from \$2 per share and maintaining her Overweight rating on the stock.

Commenting on the private placement, the analyst added that despite the potential for heavy dilution, it removes a key overhang for TSHA and allows the company to get past key catalysts for its two [lead gene therapy programs](#).

More on Taysha

- [Taysha downgraded at Jefferies citing uncertain outlook for lead asset](#)
- [Taysha falls as FDA seeks additional dosing for CNS disorder candidate](#)
- [Taysha Gene stock soars 36% as Astellas to pick 15% stake for \\$50M](#)
- [Seeking Alpha's Quant Rating on Taysha Gene Therapies](#)
- [Earnings data for Taysha Gene Therapies](#)

On the Move

AAOI, COXS and HROW are among after hour movers

Aug. 14, 2023 5:59 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | CDXS, AAOI, HROW... | By: Urvi Shah, SA News Editor

- **Gainers:** Taysha Gene Therapies (NASDAQ:TSHA) +12%.
- Arcellx (ACLX) +10%.
- Jet.AI (JTAI) +10%.
- CompoSecure (CMPO) +9%.
- Codex (CDXS) +9%.
- **Losers:** Design Therapeutics (DSGN) -43%.
- Definitive Healthcare (DH) -6%.
- Rumble (RUM) -4%.
- Harrow Health (HROW) -4%.
- Applied Optoelectronics (AAOI) -3%.

Healthcare

On the Move

Taysha gains FDA fast track status for Rett syndrome therapy

Aug. 24, 2023 8:32 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwthana, SA News Editor



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- Taysha Gene Therapies (NASDAQ:[TSHA](#)) traded ~10% higher pre-market Thursday after the FDA issued its Fast Track Designation for the biotech's gene transfer therapy TSHA-102 to treat the rare neurodevelopmental disorder Rett syndrome.

- TSHA-102 is currently undergoing a Phase 1/2 trial in Canada for adults with Rett syndrome, which is characterized by motor and respiratory impairments, among other issues.
- As a genetically-driven disorder, Rett syndrome currently has no FDA-approved disease-modifying therapies.
- The FDA offers the Fast Track designation to accelerate the development and review of drugs targeted at serious conditions with unmet medical needs. It allows developers to communicate frequently with the regulator about plans for clinical studies.
- Companies with Fast Track Designations can secure the FDA's Accelerated Approval and Priority Review, subject to conditions, enabling patients to access those treatments sooner.
- Having received FDA clearance to start a trial for children, the company intends to dose the first pediatric patient [with TSHA-102 in Q1 2024](#).

More on Taysha

- [Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch](#)
- [Taysha surges on private placement, early data from lead program](#)
- [Taysha Gene Therapies agrees on ~\\$150M private placement financing](#)
- [Taysha downgraded at Jefferies citing uncertain outlook for lead asset](#)
- [Taysha falls as FDA seeks additional dosing for CNS disorder candidate](#)
- [Seeking Alpha's Quant Rating on Taysha Gene Therapies](#)

Healthcare

On the Move

Taysha Gene Therapies climbs after regaining Nasdaq compliance

Aug. 29, 2023 10:19 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Dulan Lokuwithana, SA News Editor



GOCMEN/iStock via Getty Images

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) traded higher in the morning hours Tuesday after the company said it regained compliance with certain requirements of the Nasdaq stock market.
- According to notifications received from Nasdaq on Monday, the gene therapy developer regained compliance with listing rules 5450(b)(2)(A) and 5450((a))(1), which deal with minimum market value and minimum bid price, respectively.
- Both matters are now closed, Taysha ([TSHA](#)) said in a regulatory filing before the market opened on Tuesday.

- The announcement has excited the company's investors, with its shares adding ~18%, the biggest intraday for the year after an over two-fold rise the company recorded early this month following a ~\$150M private placement.
- Taysha ([TSHA](#)) is a favorite on Wall Street with eight Buy ratings, a Hold rating, and no Sell ratings.

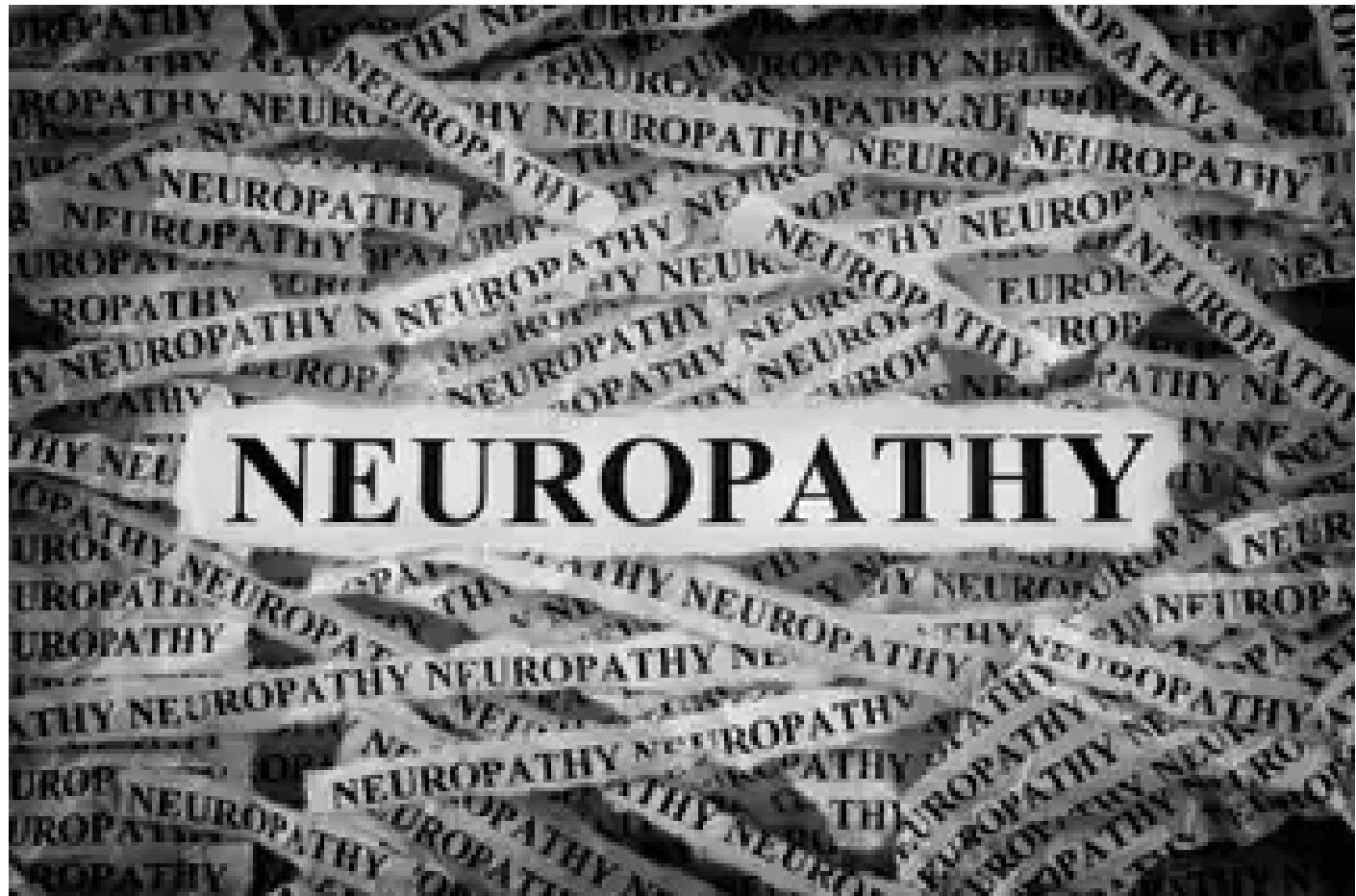
More on Taysha

- [Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch](#)
- [Taysha gains FDA fast track status for Rett syndrome therapy](#)
- [Taysha surges on private placement, early data from lead program](#)
- [Taysha downgraded at Jefferies citing uncertain outlook for lead asset](#)
- [Seeking Alpha's Quant Rating on Taysha Gene Therapies](#)

Healthcare

Taysha Gene Therapies ceases development of neurology drug TSHA-120

Sep. 19, 2023 5:41 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Val Brickates Kennedy, SA News Editor | 1 Comment



Professor25/iStock via Getty Images

Taysha Gene Therapies (NASDAQ:[TSHA](#)) is discontinuing development of its drug candidate TSHA-120 for the treatment of giant axonal neuropathy due to problems designing a study that could support a potential FDA market application.

The biotech group said it is pursuing external strategic options for the program that could enable further development of the drug. It added that Astellas Gene Therapies has elected not to exercise its option to obtain an exclusive license to TSHA-120.

Taysha said discontinuation of the program will extend its cash runway into Q4 2025 and will help support the development of TSHA-102 for the treatment of Rett syndrome.

TSHA-120 was in Phase 2 testing, according to Taysha's website.

More on Taysha Gene Therapies

- [Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch](#)
- [Taysha Gene Therapies climbs after regaining Nasdaq compliance](#)
- [Taysha gains FDA fast track status for Rett syndrome therapy](#)
- [Taysha surges on private placement, early data from lead program](#)
- [Taysha Gene Therapies agrees on ~\\$150M private placement financing](#)

On the Move

Biggest stock movers today: Bausch Health, Pinterest, Instacart, Coty and more

Sep. 20, 2023 9:35 AM ET | ARS Pharmaceuticals, Inc. (SPRY) Stock, TSHA Stock, CART Stock, COTY Stock, PINS Stock, BHC Stock, BHC:CA Stock | By: Niloofer Shaikh, SA News Editor



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Stock futures inched higher on Wednesday as market players prepare for the second day of the Fed's policy meeting.

Here are some of Wednesday's biggest stock movers:

Biggest stock gainers

Shares of Bausch Health (NYSE:BHC) shares **rose 6%** after Jefferies upgraded the stock from hold to buy, lifting its price target to \$16 from \$9 before, citing strong Q3 earnings, improved clarity on the Bausch + Lomb spinoff, and potential legal victories.

Pinterest's (NYSE:PINS) stock **rose over 5%** as several Wall Street firms **expressed positive sentiment** after the company's investor day. Citi analyst Ronald Josey upgraded Pinterest to buy from neutral and raised his price target to \$36. He believes engagement can continue to rise, the company's advertising approach is improving monetization, and adjusted EBITDA margins will continue to rise. D.A. Davidson analyst Tom Forte also upgraded Pinterest to buy from neutral and raised the price target to \$35, citing the impact of the company's long-term adjusted EBITDA forecast. J.P. Morgan analyst Doug Anmuth raised his estimates and price target on Pinterest, noting the event was "solid" and the focus on product innovation and a lower ad funnel were good news.

Coty (NYSE:COTY) shares **increased by more than 5%** after the company upgraded its first-half and full-year forecasts. The company **predicts core LFL sales growth of +10 to +12%** in 1H24, up from an earlier estimate of +8 to +10%, and core LFL sales growth of +8 to +10% in FY2024. The company continues to target modest gross margin expansion in FY24 and adjusted EBITDA margin expansion of 10 bps to 30 bps, indicating adjusted EBITDA of roughly \$1.08B to \$1.09B vs. at current FX rates, an increase from the previous estimate of \$1.07B to \$1.08B.

Biggest stock losers

After an exciting debut, the stock price of grocery delivery service Instacart (NASDAQ:CART) dropped **more than 5%** in Wednesday's premarket trade. The share price of the stock increased by as much as 43% on Tuesday before declining to **end the day 12.3% higher at \$33.70**. The IPO, which raised \$660M at a price of \$30 per share, opened at \$42 per share.

Shares of Taysha Gene Therapies (NASDAQ:TSHA) **slumped over 9%** after the biotech firm announced the **discontinuation of the development of its drug candidate TSHA-120** for the treatment of giant axonal neuropathy due to design issues. The biotech group is exploring external strategic options for further drug development. Astellas Gene Therapies has not exercised its exclusive license option for TSHA-120. The discontinuation will extend the company's cash runway until Q4 2025.

ARS Pharmaceuticals (NASDAQ:[SPRY](#)) shares **lost more than 50%** after the FDA **declined** to approve its epinephrine nasal spray, neffy, as a treatment for Type I allergic reactions in adults and children. The FDA requested additional data from a study on the performance of repeat doses of neffy against epinephrine injection in allergen-induced allergic rhinitis conditions. This decision comes against the recommendation of an FDA Advisory Committee in May 2023 that endorsed the marketing authorization without requiring additional data on safety and efficacy. ARS Pharma plans to resubmit a new drug application in H1 2024.

Related stories:

- [S&P, Nasdaq, Dow futures tick up, yields ease with all eyes on Fed forecasts](#)
- [Pinterest: Unlocking Growth](#)
- [Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch](#)
- [U.S. Recession Probability Starts To Recede](#)
- [Instacart: Likely Unsustainable Profits And Expensive Valuation](#)

Earnings News

Taysha Gene Therapies GAAP EPS of -\$0.93 misses by \$0.76, revenue of \$4.74M beats by \$2.64M

Nov. 14, 2023 4:23 PM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Urvi Shah, SA News Editor

- Taysha Gene Therapies [press release](#) (NASDAQ:[TSHA](#)): Q3 GAAP EPS of -\$0.93 **misses by \$0.76**.
- Revenue of \$4.74M **beats by \$2.64M**.

More on Taysha Gene Therapies

- [Taysha Gene Therapies: TSHA-102's Potential Is A 'Buy'](#)
- [Taysha Gene Therapies: Rett Syndrome Data Makes This A Must Watch](#)
- [Biggest stock movers today: Bausch Health, Pinterest, Instacart, Coty and more](#)
- [Taysha Gene Therapies ceases development of neurology drug TSHA-120](#)
- [Seeking Alpha's Quant Rating on Taysha Gene Therapies](#)

[Healthcare](#)[On the Move](#)

Taysha Gene Therapies says Manning buys 100K shares through indirect interest in co

Nov. 20, 2023 11:41 AM ET | **Taysha Gene Therapies, Inc. (TSHA) Stock** | By: Sinchita Mitra, SA News Editor

- Taysha Gene Therapies (NASDAQ:[TSHA](#)) disclosed that Paul Manning bought 100K shares indirectly in the company at \$1.63 apiece on Nov. 17.
- Shares in the company up [6.76%](#).

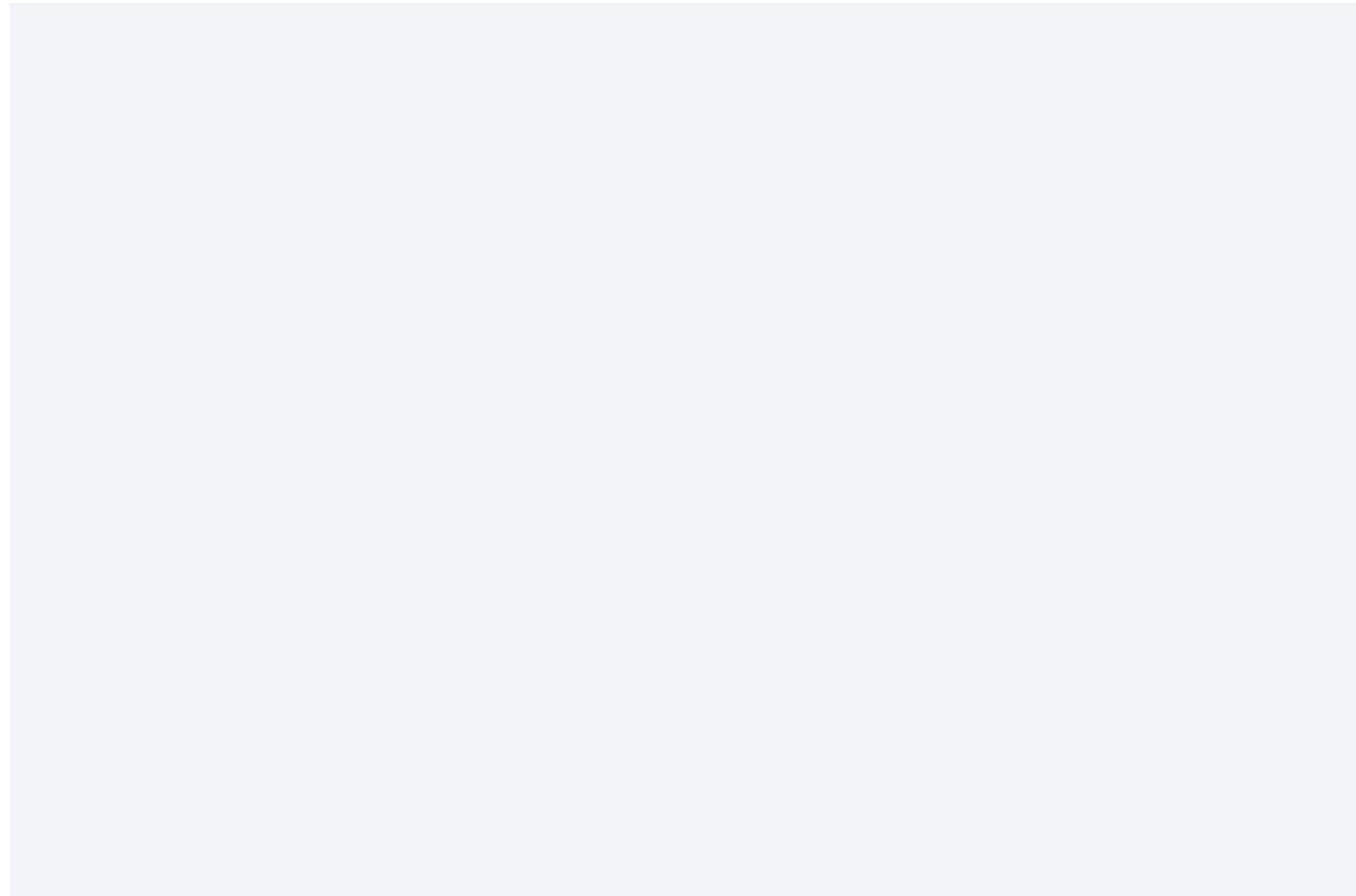
More on Taysha Gene Therapies

- [Taysha Gene Therapies: TSHA-102's Potential Is A 'Buy'](#)
- [Taysha Gene Therapies ceases development of neurology drug TSHA-120](#)

[Financials](#)[Healthcare](#)

Trinity Capital provides \$40M term loan to Taysha Gene Therapies

Dec. 19, 2023 7:43 AM ET | **Trinity Capital Inc. (TRIN), TSHA Stock** | By: Jaskiran Singh, SA News Editor | 1 Comment



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- Trinity Capital (NASDAQ:TRIN) Tuesday announced a commitment of \$40M in term loans to Taysha Gene Therapies (NASDAQ:TSHA).
- With the term loan, Taysha believes it will be able to fund its operating expenses and capital requirements into 2026 to support the clinical development of its TSHA-102 program in Rett syndrome.

- Taysha is focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system.
- Its lead clinical program TSHA-102 is in evaluation for Rett syndrome, a rare neurodevelopmental disorder with no approved disease-modifying therapies that treat the root cause of the disease.
- Source: [Press Release](#)

More on Trinity Capital, Taysha Gene Therapies, etc.

- [Trinity Capital: Top 4 High Dividend Stock, 15% Yield, Record Q3 Earnings](#)
- [Taysha Gene Therapies, Inc. \(TSHA\) Q3 2023 Earnings Call Transcript](#)
- [Trinity Capital: 13.6% Yield, Healthy Financials, Still A Buy?](#)
- [Trinity Capital provides \\$45 million growth capital to Neurolens](#)
- [Trinity Capital Q3 2023 Earnings Preview](#)

Health Care Sector Update for 10/24/2023: MEDP, CTRX, TSHA

October 24, 2023 — 01:52 pm EDT

Written by MT Newswires for [MTNewswires ->](#)

Health care stocks were mixed Tuesday afternoon, with the NYSE Health Care Index down 0.1% and the Health Care Select Sector SPDR Fund (XLV) up 0.1%.

The iShares Biotechnology ETF (IBB) rose 0.9%.

In company news, Medpace ([MEDP](#)) shares jumped 17% after Baird upgraded the stock to outperform from neutral and raised its price target to \$289 from \$270 following Q3 results.

Citius Pharmaceuticals (CTXR) and blank-check firm TenX Keane Acquisition (TENKU) said they have signed an agreement for a merger of TenX and Citius' oncology subsidiary, which will be listed on Nasdaq. Citius Pharmaceuticals gained 14%.

Taysha Gene Therapies ([TSHA](#)) said that new preclinical in vitro data reinforced the potential of its TSHA-102 therapy to address the root cause of Rett syndrome. Its shares rose 2.3%.

3 Best Stocks to Buy Now, 9/21/2023, According to Top Analysts

September 21, 2023 — 06:10 am EDT

Written by Radhika Saraogi for [TipRanks ->](#)

Which stocks are best to buy now? According to Top Wall Street Analysts, the three stocks listed below are Strong Buys. Each stock received a new Buy rating recently and has a significant upside as well.

To find more stocks like these, take a look at TipRanks' [Analyst Top Stocks tool](#). It shows you a real-time list of all stocks that have been recently rated by Top-ranking Analysts.

Here are today's top stock picks, according to analysts. Click on any ticker to thoroughly research the stock before you decide whether to add it to your portfolio.

Portillo's ([NASDAQ:PTLO](#)) – This American restaurant chain is known for its classic Chicago-style menu. Yesterday, [Bank of America Securities analyst Sara Senatore](#) maintained a Buy rating on the stock with a price target of \$28. All four Top Analysts who recently rated the stock gave it a Buy. Collectively, their 12-month price targets imply an upside of nearly 57%.

Taysha Gene Therapies ([NASDAQ:TSHA](#)) – Taysha is a clinical-stage biotechnology company focused on developing gene therapies for severe diseases of the central nervous system. Yesterday, [Canaccord Genuity analyst Whitney Ijem](#) reaffirmed a Buy rating on the stock with a price target of \$6. TSHA stock has received Buy recommendations from all four Top Analysts who have recently rated it. Overall, the consensus 12-month price target suggests an increase of about 80%.

Marvell Technology ([NASDAQ:MRVL](#)) – Marvell offers data infrastructure semiconductor solutions for computing, networking, security, and storage purposes. Yesterday, [Rosenblatt Securities analyst Hans Mosesmann](#) reiterated a Buy rating on the stock with a price target of \$100. Interestingly, 18 out of 19 Top Analysts who recently rated the stock gave it a Buy. Taken together, their 12-month price targets imply an upside of over 38%.

Truist Securities Maintains Taysha Gene Therapies (TSHA) Buy Recommendation

September 20, 2023 — 09:08 pm EDT

Written by George Maybach for [Fintel ->](#)

Fintel reports that on September 20, 2023, Truist Securities [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 79.04% Upside

As of August 31, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.84. The forecasts range from a low of 2.02 to a high of \$8.40. The average price target represents an increase of 79.04% from its latest reported closing price of 3.26.

See our [leaderboard of companies](#) with the largest price target upside.

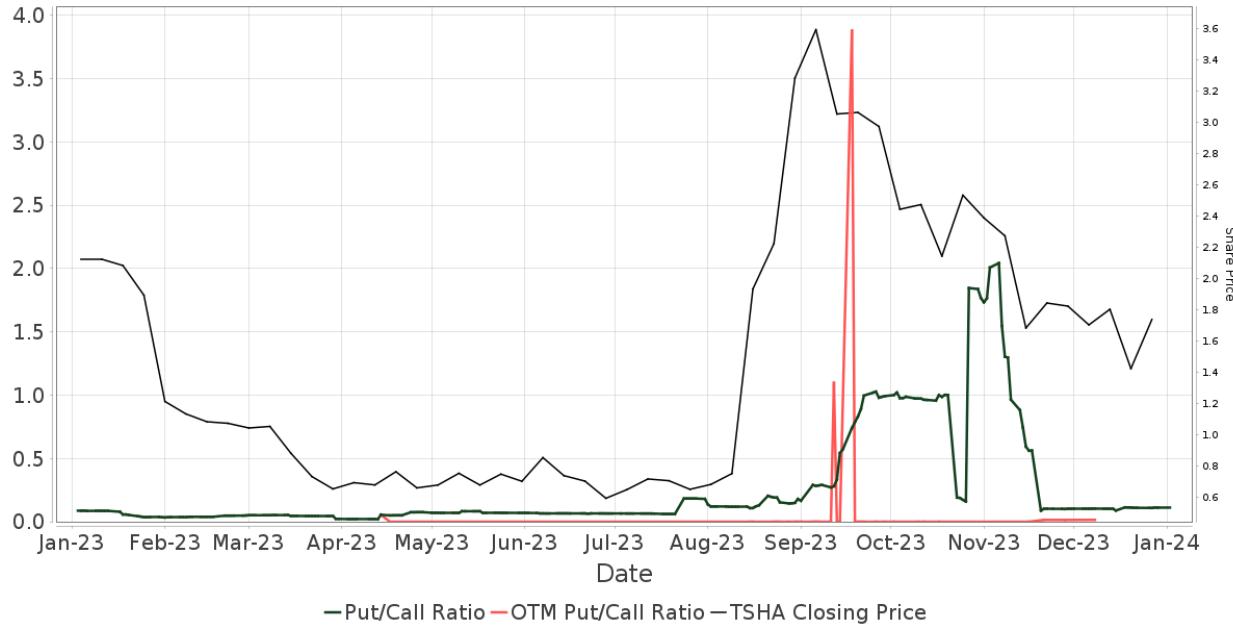
The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [63 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 19 owner(s) or 23.17% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, an increase of 2.12%. Total shares owned by institutions decreased in the last three months by 18.80% to 14,165K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io

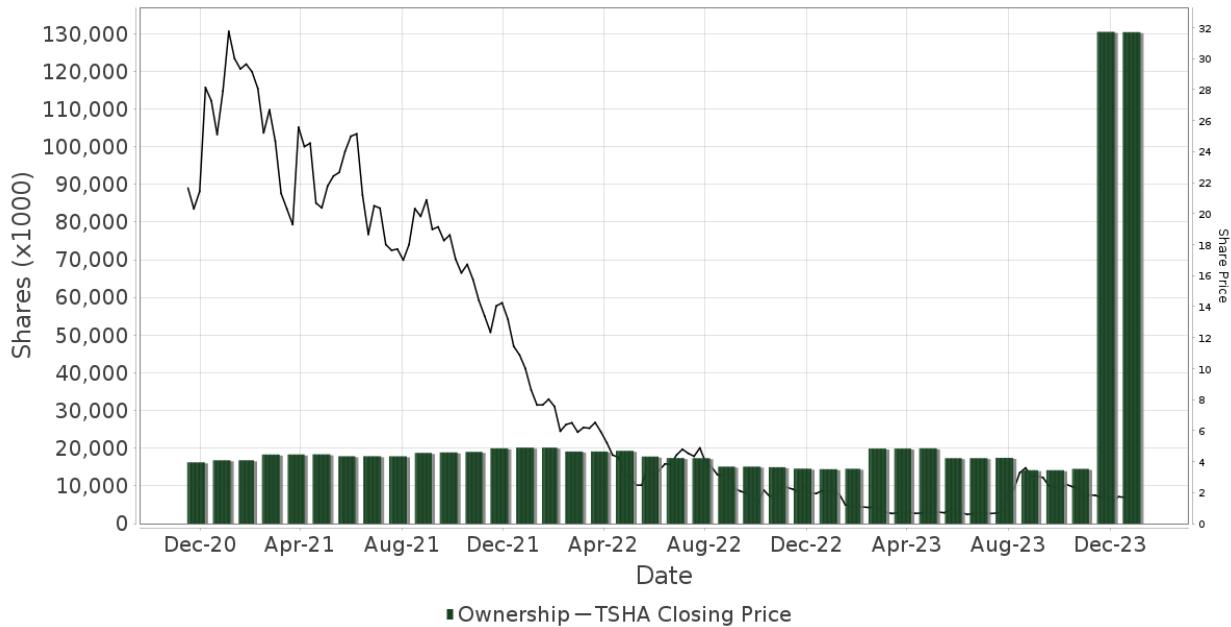


The [put/call ratio](#) of TSHA is 0.79, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 0.80% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 0.77% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 0.67% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a **decrease** of 37.67%. The firm **decreased** its portfolio allocation in TSHA by 22.73% over the last quarter.

[Laurion Capital Management](#) holds 1,172K shares representing 0.63% ownership of the company. In its prior filing, the firm reported owning 1,293K shares, representing a **decrease** of 10.31%. The firm **decreased** its portfolio allocation in TSHA by 38.22% over the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 0.62% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's

proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Canaccord Genuity Maintains Taysha Gene Therapies (TSHA) Buy Recommendation

September 20, 2023 — 09:08 pm EDT

Written by George Maybach for [Fintel ->](#)

Fintel reports that on September 20, 2023, Canaccord Genuity [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

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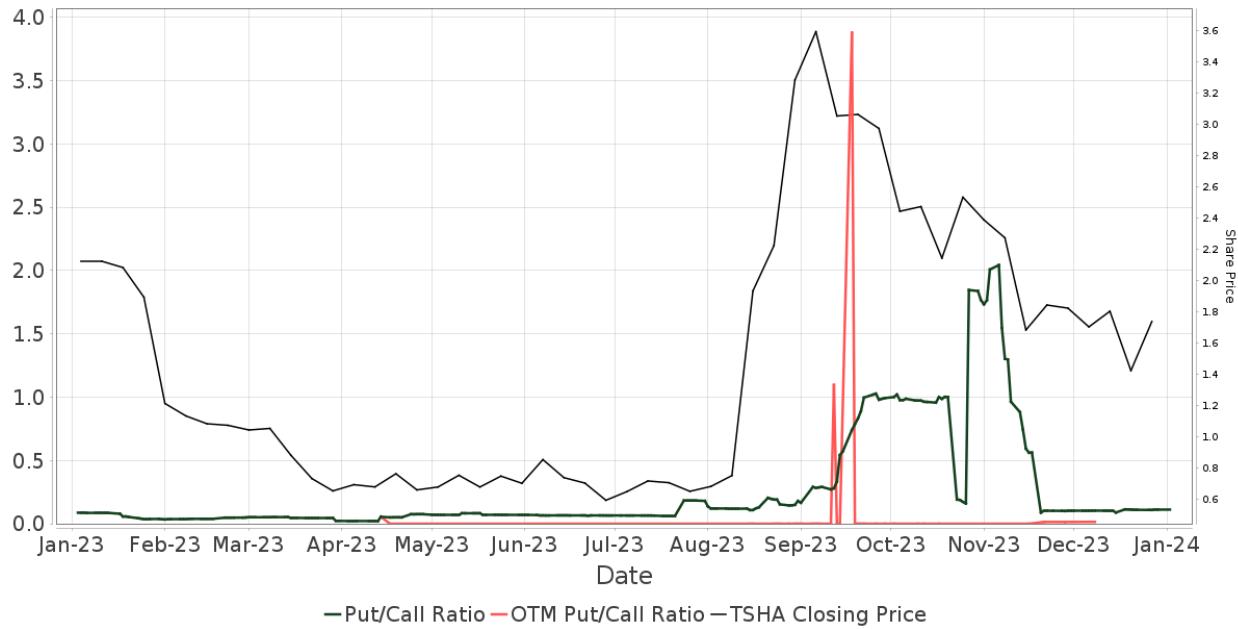
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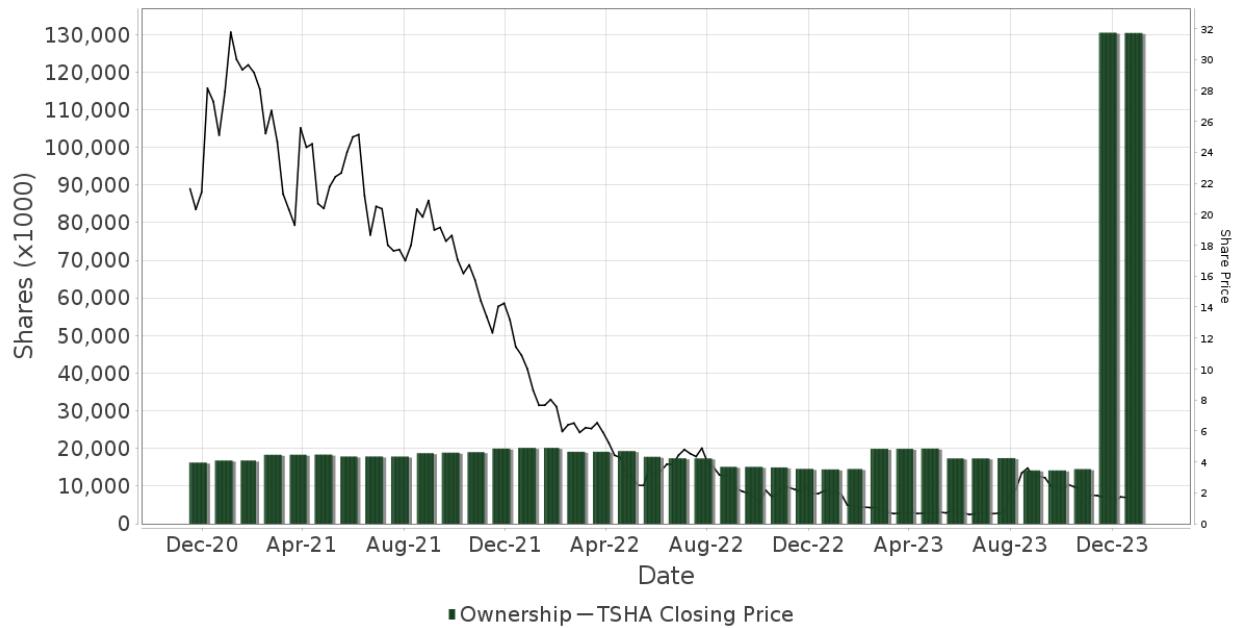


The [put/call ratio](#) of TSHA is 0.79, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



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Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Needham Reiterates Taysha Gene Therapies (TSHA) Buy Recommendation

September 20, 2023 — 09:08 pm EDT

Written by George Maybach for [Fintel](#) ->

Fintel reports that on September 20, 2023, Needham [reiterated](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 79.04% Upside

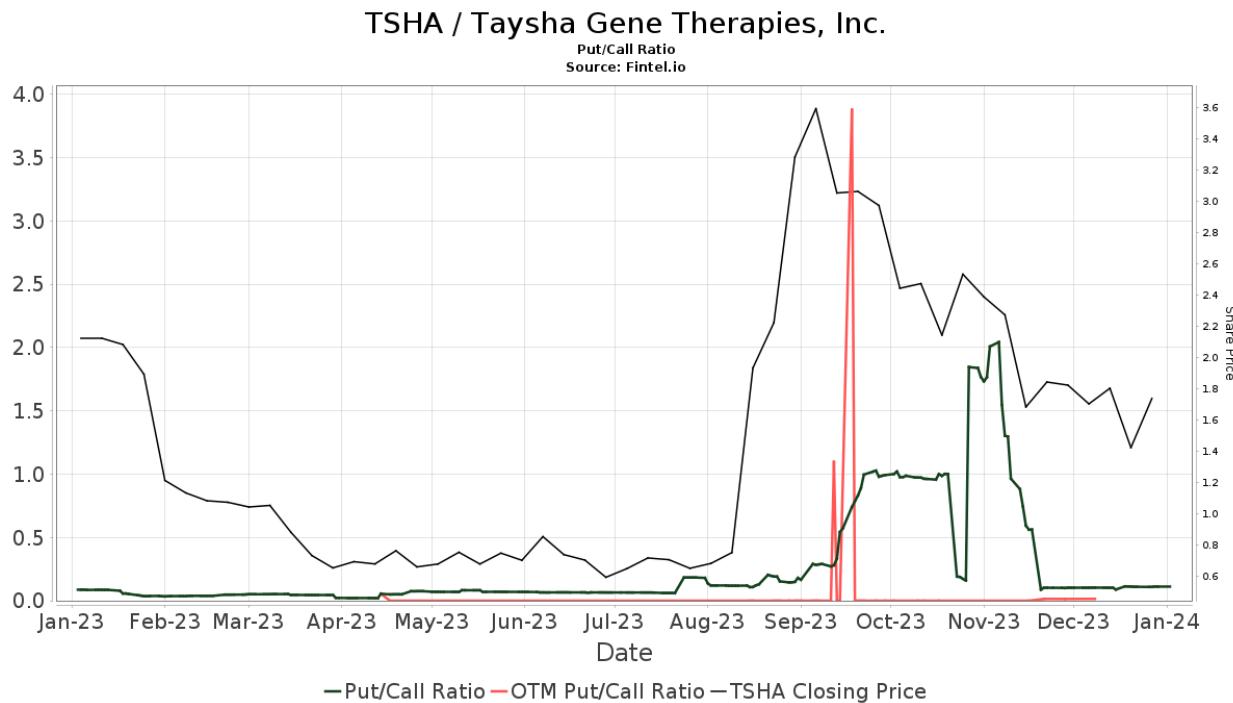
As of August 31, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.84. The forecasts range from a low of 2.02 to a high of \$8.40. The average price target represents an increase of 79.04% from its latest reported closing price of 3.26.

See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [63 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 19 owner(s) or 23.17% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, an increase of 2.12%. Total shares owned by institutions decreased in the last three months by 18.80% to 14,165K shares.

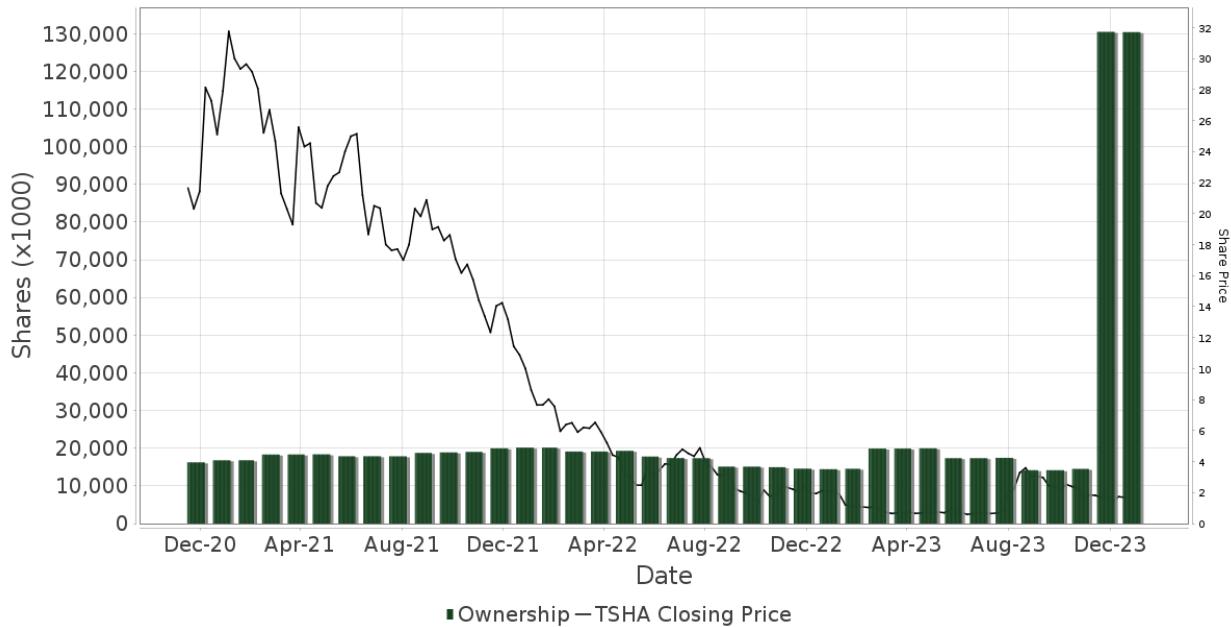


The [put/call ratio](#) of TSHA is 0.79, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 0.80% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 0.77% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 0.67% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a **decrease** of 37.67%. The firm **decreased** its portfolio allocation in TSHA by 22.73% over the last quarter.

[Laurion Capital Management](#) holds 1,172K shares representing 0.63% ownership of the company. In its prior filing, the firm reported owning 1,293K shares, representing a **decrease** of 10.31%. The firm **decreased** its portfolio allocation in TSHA by 38.22% over the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 0.62% ownership of the company. No change in the last quarter.

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Taysha Discontinues Development Of TSHA-120 In Giant Axonal Neuropathy

September 19, 2023 — 04:48 pm EDT

Written by RTTNews.com for [RTTNews ->](#)

(RTTNews) - Clinical-stage gene therapy company Taysha Gene Therapies, Inc. (TSHA), Tuesday announced that the company will discontinue the development of its TSHA-120 program in evaluation for the treatment of giant axonal neuropathy (GAN).

The decision was based on the receipt of Type C meeting feedback from the FDA regarding a registrational path for TSHA-120.

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Prefabricated Cabins

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Further, Taysha announced that Astellas Gene Therapies, Inc. has elected not to exercise its option to obtain an exclusive license to TSHA-120 under the Option Agreement between Astellas and Taysha.

"We believe we have made significant progress in demonstrating the therapeutic potential of TSHA-120 and identifying a potential registrational path. Following FDA feedback, we have made the decision to discontinue further development of the program due to challenges related to the feasibility of the study designs to support a

potential BLA submission in this ultra-rare neurodegenerative disease," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha.

2 Healthcare Stocks That More Than Tripled Recently. Can They Keep Climbing?

September 19, 2023 — 06:41 am EDT

Written by Cory Renauer for [The Motley Fool](#) ->

From Aug. 11 through Sept. 15, shares of the troubled Canadian cannabis producer **Canopy Growth** ([NASDAQ: CGC](#)) rocketed 235% higher. Shares of clinical-stage drugmaer **Taysha Gene Therapies** ([NASDAQ: TSHA](#)) soared an even more impressive 345% over the same time frame.

Are there more eye-popping gains up ahead, or is it already too late to buy these volatile stocks? To find out, let's look at the catalysts that drove them higher.



Image source: Getty Images.

1. Canopy Growth

Investors cheered Canopy Growth's recent decision to cease funding its ill-fated dietary supplement business, BioSteel, to focus on selling cannabis and related products.

Top-line revenue from its dietary supplement business rocketed to CA\$32.5 million (more than \$24 million in U.S. dollars) in the fiscal first quarter from CA\$13.7 million (more than \$10 million USD) a year earlier, but this doesn't tell the whole story. The costs of producing and warehousing supplements were a lot higher than Canopy Growth was expecting, again. Before factoring in sales, general, and administrative expenses, the company lost CA\$0.24 for every dollar of BioSteel revenue recorded in the first quarter.

BioSteel's fiscal first-quarter performance was nearly twice as damaging as the negative [gross profit margin](#) it reported in the prior-year period.

Canopy Growth was right to jettison its failing supplement business, but this still isn't a good time to buy the stock. Investors should know that BioSteel isn't the only operating segment with a negative gross margin.

Canopy Growth's main operating segment, selling cannabis in products Canada, lost CA\$0.01 for every dollar of revenue it reported in the fiscal first quarter. That's a huge improvement over the negative 24% gross margin it reported a year earlier, but hardly a reason to ignore substantial doubt about the company's ability to continue as a [going concern](#) for another 12 months as stated in its latest earnings report.

Canopy Growth expects to achieve positive adjusted earnings before interest, taxes, depreciation, and amortization ([EBITDA](#)) by the end of fiscal 2024. It's probably best to wait and see if it can meet this expectation before risking any of your hard-earned money on this super-risky stock.

Taysha Gene Therapies

Taysha Gene Therapies is a clinical-stage biotechnology company developing new gene therapies for inherited neurological conditions. The stock surged after the company reported early signs of success from a phase 1 trial with its experimental Rett syndrome treatment, TSHA-102.

The first Rett syndrome patient treated showed signs of improvement during a checkup conducted four weeks after receiving a single dose of TSHA-102. Rett syndrome is a neurodevelopmental disorder often caused by a mutated MECP2 gene. TSHA-102 contains a shortened version of this gene, and it appears to work as intended.

In addition to a lack of negative safety signals, the first patient to receive TSHA-102 reported improvement on several tests of disease severity. The trial's principal investigator also observed improvements in the patient's motor skills and vocalization abilities.

Despite its stock price more than quadrupling, expectations for Taysha Gene Therapies are still fairly low. Its [market cap](#) at recent prices is just \$213 million.

Rett syndrome is rare, but it still affects roughly 350,000 people worldwide. In other words, there are more than enough addressable patients to drive annual sales of TSHA-102 past \$300 million if it continues to impress.

Taysha Gene Therapies expects to dose a second patient with TSHA-102 in the third quarter. If subsequent results fall in line with the first patient's, the stock could rocket much higher than it already has.

While Taysha stock could jump again on positive results, gene therapy trials are often unpredictable. Any negative safety signal could lead regulators to pause the company's development plans and crush its stock price in the process.

Before taking a big risk on this biotech stock, investors should know that Taysha finished June with just \$45 million in cash after burning through \$124 million over the past 12 months. Some new financing will inject \$150 million into its coffers, but it will also more than triple its number of outstanding shares.

Taysha's dilutive financing severely lowers long-term shareholders' chances of realizing future gains even though it only lengthens the company's cash runway until 2025. It's probably best to watch this stock from a safe distance until the company has a lot more clinical trial data to digest.

10 stocks we like better than Canopy Growth

When our analyst team has a stock tip, it can pay to listen. After all, the newsletter they have run for over a decade, *Motley Fool Stock Advisor*, has tripled the market.*

They just revealed what they believe are the [ten best stocks](#) for investors to buy right now... and Canopy Growth wasn't one of them! That's right -- they think these 10 stocks are even better buys.

Taysha Gene Therapies (TSHA) Price Target Increased by 11.47% to 5.84

August 31, 2023 — 08:44 am EDT

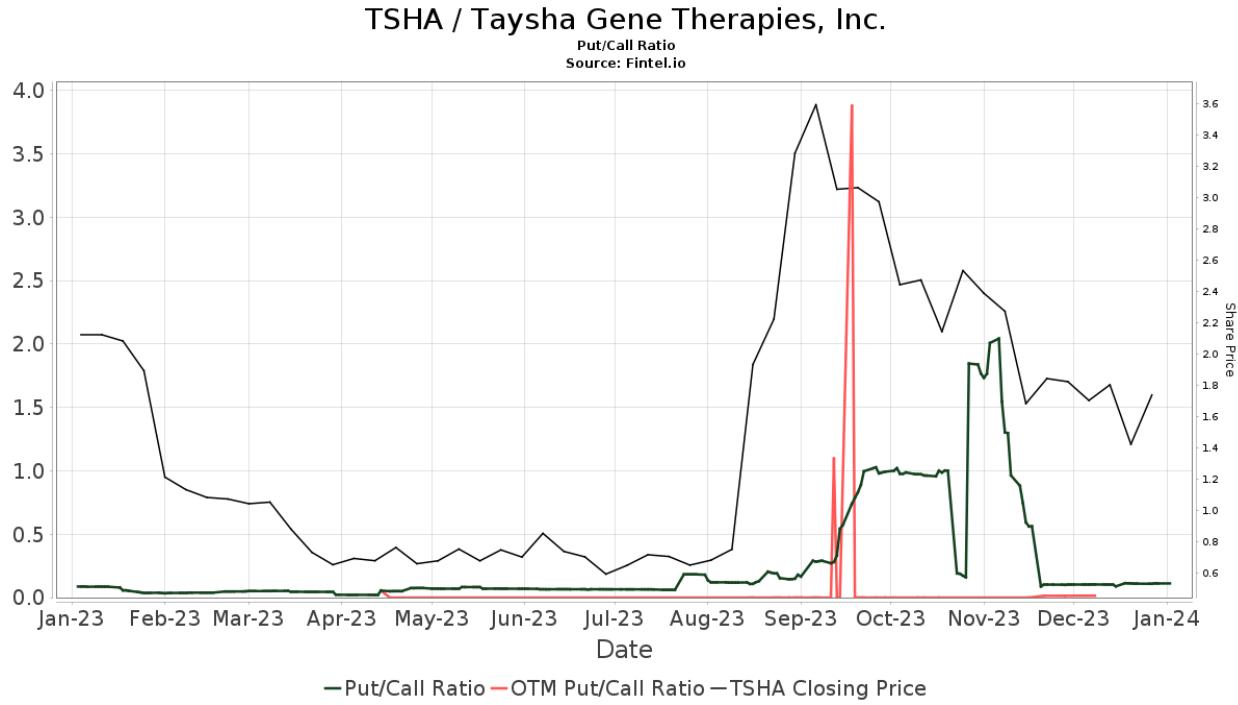
Written by George Maybach for [Fintel ->](#)

The average one-year [price target](#) for Taysha Gene Therapies ([NASDAQ:TSHA](#)) has been revised to 5.84 / share. This is an increase of 11.47% from the prior estimate of 5.24 dated August 1, 2023.

The price target is an average of many targets provided by analysts. The latest targets range from a low of 2.02 to a high of 8.40 / share. The average price target represents an increase of 77.95% from the latest reported closing price of 3.28 / share.

What is the Fund Sentiment?

There are [64 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 17 owner(s) or 20.99% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 3.90%. Total shares owned by institutions decreased in the last three months by 18.34% to 14,165K shares.

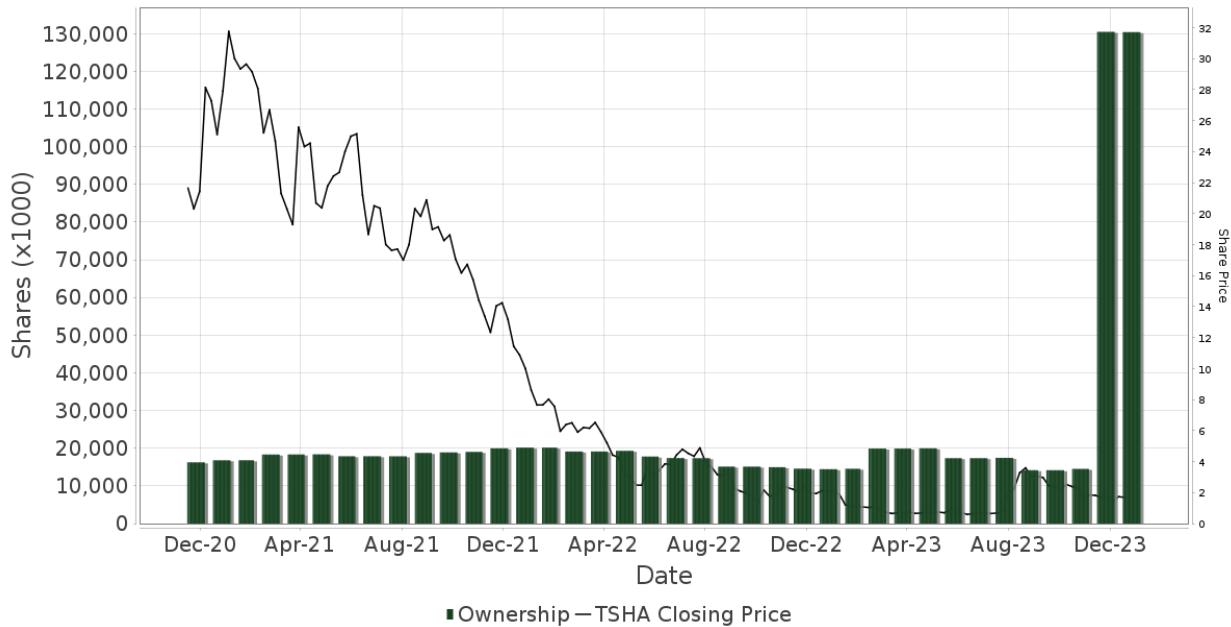


The [put/call ratio](#) of TSHA is 0.15, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 0.80% ownership of the company. No change in the last quarter.

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Additional reading:

- [JOINT FILING AGREEMENT](#)
- [Taysha Gene Therapies Announces Fast Track Designation Granted by U.S. FDA for TSHA-102 in Rett Syndrome Fast Track Designation \(FTD\) is designed to accelerate the development and expedite the review of therapies with potential to address unmet medical needs](#)
- [JOINT FILING AGREEMENT](#)

Pre-Market Most Active for Aug 24, 2023 : SQQQ, TQQQ, NVDA, BETR, TSHA, PLTR, TSLA, AMC, NIO, CNDA, IONQ, AI

August 24, 2023 — 08:29 am EDT

The [NASDAQ 100 Pre-Market Indicator](#) is up 143.42 to 15,291.48. The total Pre-Market volume is currently 30,508,496 shares traded.

The following are the [most active stocks for the pre-market session](#):

ProShares UltraPro Short QQQ ([SQQQ](#)) is -0.55 at \$18.48, with 3,803,732 shares traded. This represents a 12.82% increase from its 52 Week Low.

ProShares UltraPro QQQ ([TQQQ](#)) is +1.16 at \$40.94, with 3,079,971 shares traded. This represents a 154.29% increase from its 52 Week Low.

NVIDIA Corporation ([NVDA](#)) is +32.32 at \$503.48, with 2,866,607 shares traded. Over the last four weeks they have had 4 up revisions for the earnings forecast, for the fiscal

quarter ending Oct 2023. The consensus EPS forecast is \$2.02. As reported by Zacks, the current mean recommendation for [NVDA](#) is in the "buy range".

Better Home & Finance Holding Company ([BETR](#)) is unchanged at \$2.09, with 2,647,188 shares traded.

Taysha Gene Therapies, Inc. ([TSHA](#)) is +0.21 at \$2.43, with 1,220,743 shares traded. As reported in the last short interest update the days to cover for [TSHA](#) is 7.34132; this calculation is based on the average trading volume of the stock.

Palantir Technologies Inc. ([PLTR](#)) is +0.53 at \$15.83, with 1,169,720 shares traded. Over the last four weeks they have had 3 up revisions for the earnings forecast, for the fiscal quarter ending Mar 2024. The consensus EPS forecast is \$0.03. [PLTR's](#) current last sale is 126.64% of the target price of \$12.5.

Tesla, Inc. ([TSLA](#)) is +3.44 at \$240.30, with 1,139,398 shares traded. [TSLA's](#) current last sale is 91.54% of the target price of \$262.5.

AMC Entertainment Holdings, Inc. ([AMC](#)) is -2.25 at \$17.35, with 777,193 shares traded., following a 52-week high recorded in prior regular session.

NIO Inc. ([NIO](#)) is +0.16 at \$11.00, with 723,104 shares traded.[NIO](#) is scheduled to provide an earnings report on 8/29/2023, for the fiscal quarter ending Jun2023. The consensus earnings per share forecast is -0.36 per share, which represents a -25 percent increase over the EPS one Year Ago

Concord Acquisition Corp II ([CNDA](#)) is +0.015 at \$10.35, with 350,126 shares traded.

IonQ, Inc. ([IONQ](#)) is +0.28 at \$16.38, with 334,657 shares traded. [IONQ's](#) current last sale is 91% of the target price of \$18.

C3.ai, Inc. ([AI](#)) is +1.2 at \$33.80, with 278,229 shares traded. [AI's](#) current last sale is 125.19% of the target price of \$27.

Thursday 8/17 Insider Buying Report: PGEN, TSHA

August 17, 2023 — 10:33 am EDT

Written by BNK Invest for [BNK Invest ->](#)

Bargain hunters are wise to pay careful attention to insider buying, because although there are many various reasons for an insider to sell a stock, presumably the only reason they would use their hard-earned cash to make a purchase, is that they expect to make money. Today we look at two noteworthy recent insider buys.

On Wednesday, Precigen's Randal J. Kirk, made a \$757,612 purchase of PGEN, buying 500,000 shares at a cost of \$1.52 each. Kirk was up about 9.5% on the buy at the high point of today's trading session, with PGEN trading as high as \$1.66 in trading on Thursday. Precigen is trading up about 8.1% on the day Thursday.

And also on Wednesday, Director John A. Stalfort III bought \$700,000 worth of Taysha Gene Therapies, buying 777,778 shares at a cost of \$0.90 a piece. This buy marks the first one filed by Stalfort III in the past twelve months. Taysha Gene Therapies is trading up about 4.1% on the day Thursday. Stalfort III was up about 153.3% on the purchase at the high point of today's trading session, with TSHA trading as high as \$2.28 in trading on Thursday.



[**VIDEO: Thursday 8/17 Insider Buying Report: PGEN, TSHA**](#)

Canaccord Genuity Maintains Taysha Gene Therapies (TSHA) Buy Recommendation

August 17, 2023 — 12:07 am EDT

Written by George Maybach for [Fintel ->](#)

Fintel reports that on August 16, 2023, Canaccord Genuity [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](https://www.nasdaq.com/symbol/TSHA)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 171.29% Upside

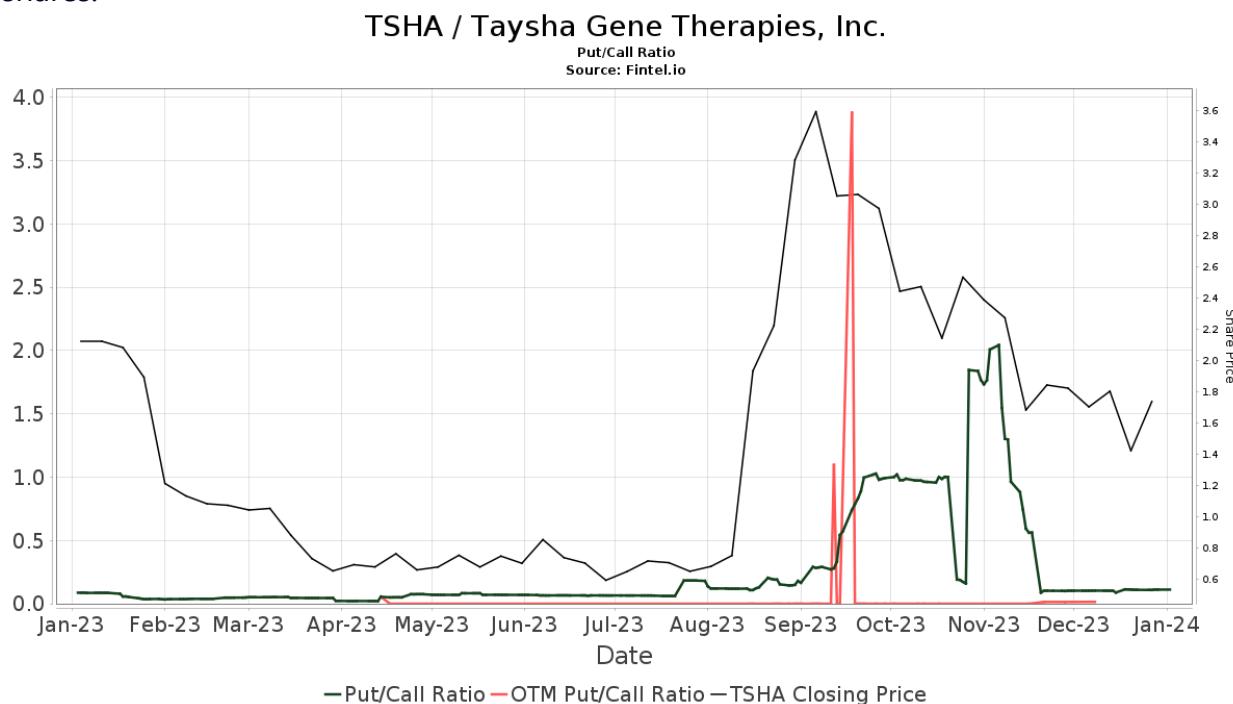
As of August 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 171.29% from its latest reported closing price of 1.93.

See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [69 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 14 owner(s) or 16.87% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 7.30%. Total shares owned by institutions decreased in the last three months by 18.63% to 14,154K shares.



The [put/call ratio](#) of TSHA is 0.11, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 1.95% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a **decrease** of 37.67%. The firm **decreased** its portfolio allocation in TSHA by 22.73% over the last quarter.

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Wednesday Sector Laggards: Biotechnology, Trucking Stocks

August 16, 2023 — 12:09 pm EDT

Written by BNK Invest for [BNK Invest ->](#)

In trading on Wednesday, biotechnology shares were relative laggards, down on the day by about 2.2%. Helping drag down the group were shares of Phenomex, down about 16.6% and shares of Taysha Gene Therapies off about 15.5% on the day.

Also lagging the market Wednesday are trucking shares, down on the day by about 2.1% as a group, led down by Yellow, trading lower by about 29% and J.B. Hunt Transport Services, trading lower by about 2.2%.

Needham Maintains Taysha Gene Therapies (TSHA) Buy Recommendation

August 15, 2023 — 11:19 pm EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on August 15, 2023, Needham [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 140.18% Upside

As of August 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 140.18% from its latest reported closing price of 2.18.

See our [leaderboard of companies](#) with the largest price target upside.

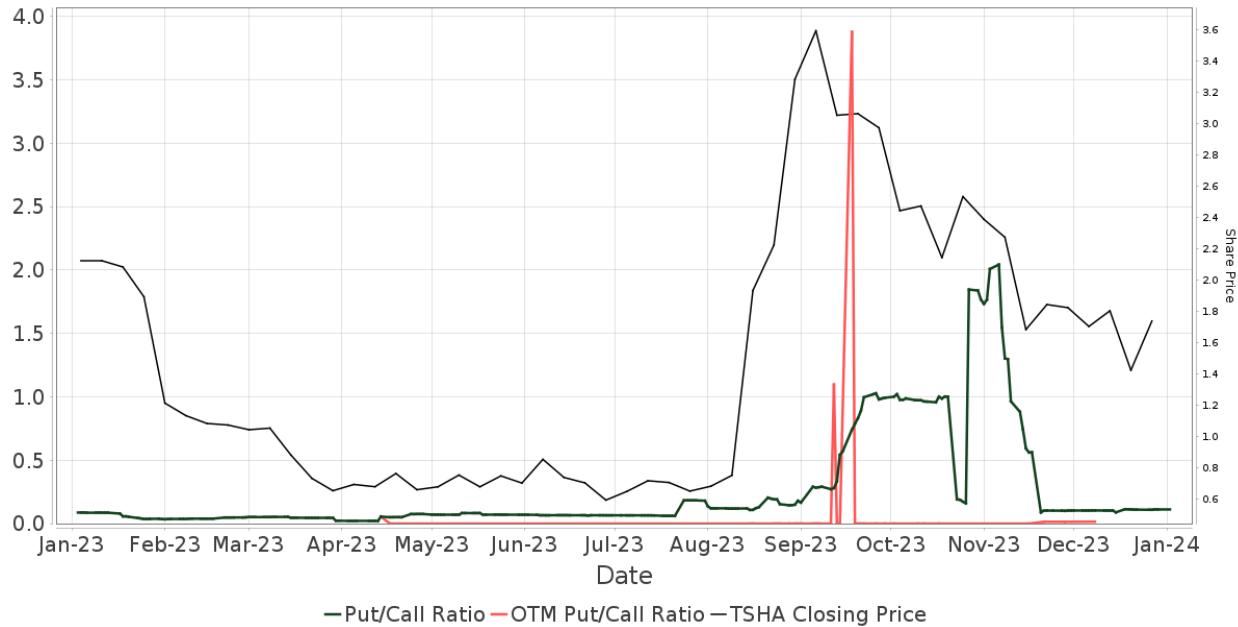
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What is the Fund Sentiment?

There are [69 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 15 owner(s) or 17.86% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 4.40%. Total shares owned by institutions decreased in the last three months by 17.87% to 14,287K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io

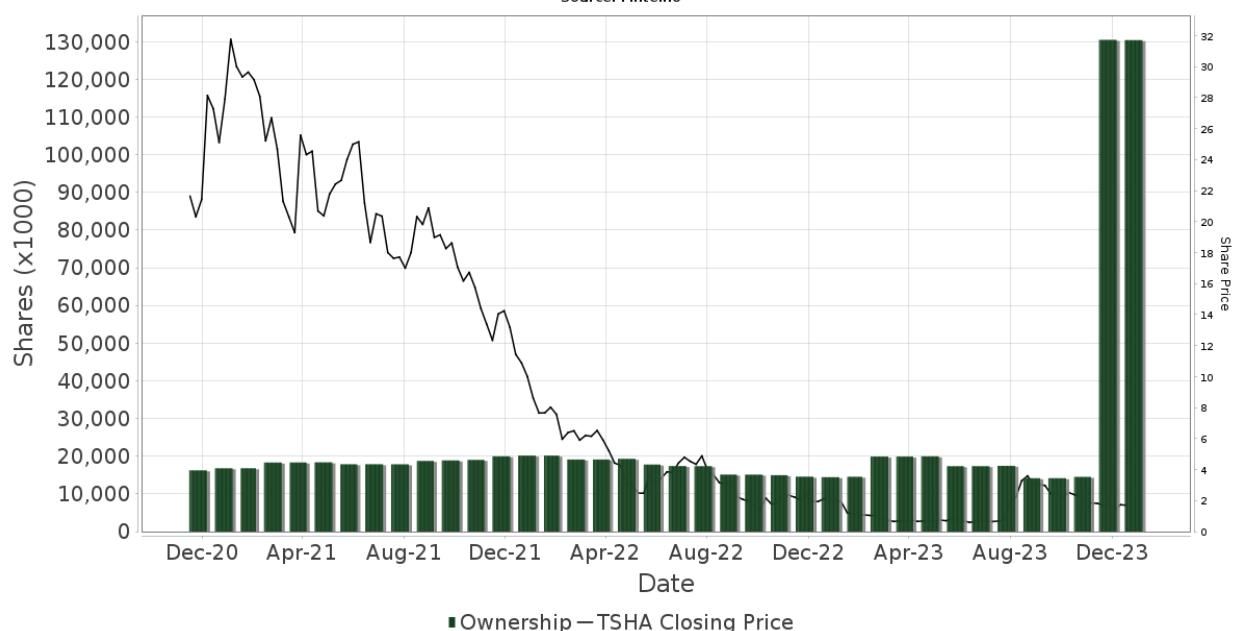


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Chardan Capital Reiterates Taysha Gene Therapies (TSJA) Buy Recommendation

August 15, 2023 — 11:19 pm EDT

Written by George Maybach for [Fintel](#) ->

Fintel reports that on August 15, 2023, Chardan Capital [reiterated](#) coverage of Taysha Gene Therapies ([NASDAQ:TSJA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 140.18% Upside

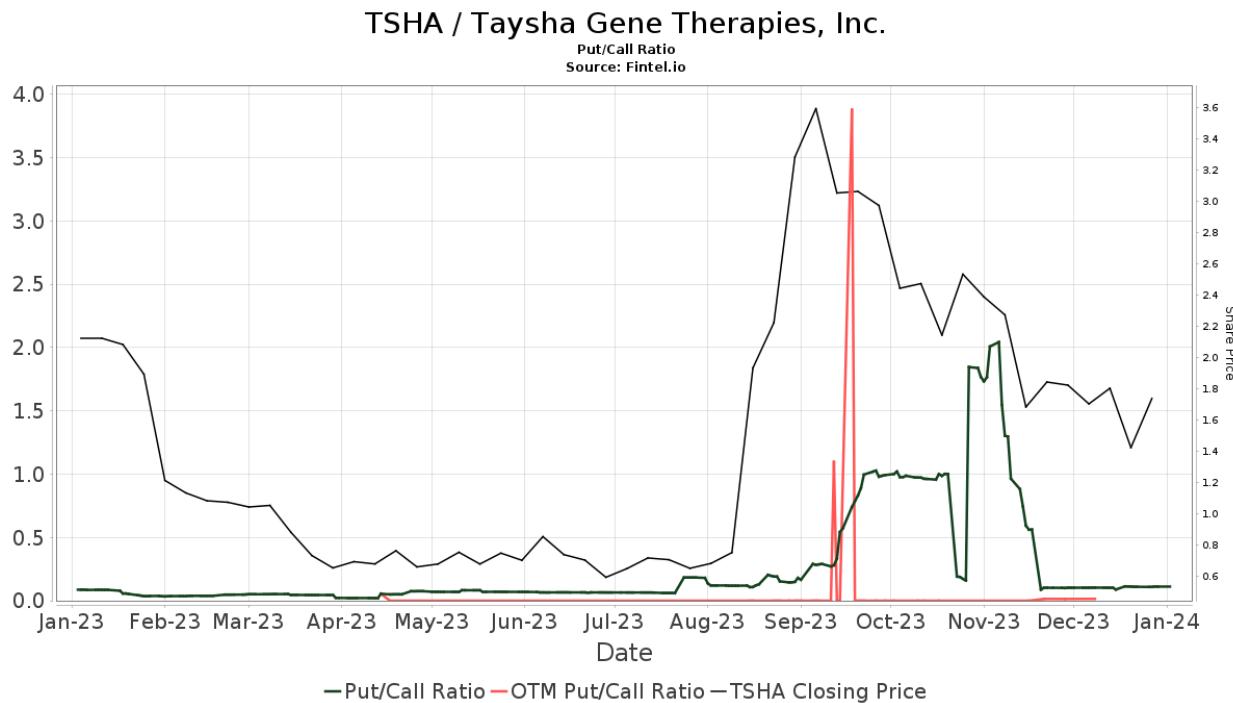
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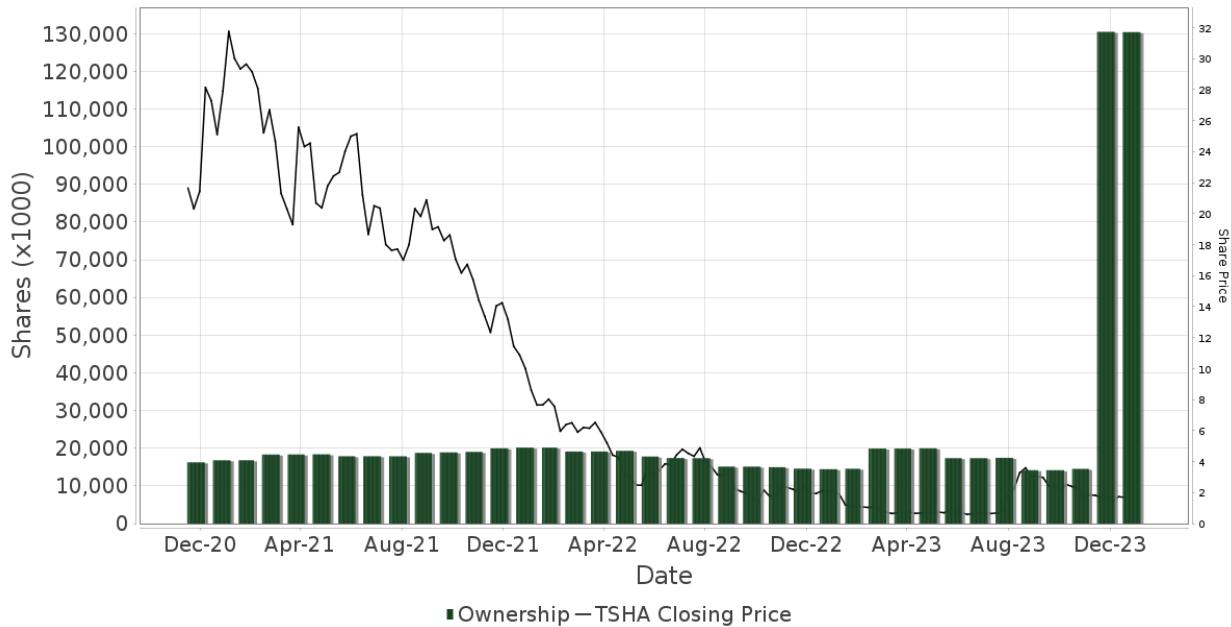


The [put/call ratio](#) of TSHA is 0.12, indicating a bullish outlook.

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Source: Fintel.io



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Truist Securities Maintains Taysha Gene Therapies (TSHA) Buy Recommendation

August 15, 2023 — 11:19 pm EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on August 15, 2023, Truist Securities [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

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See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

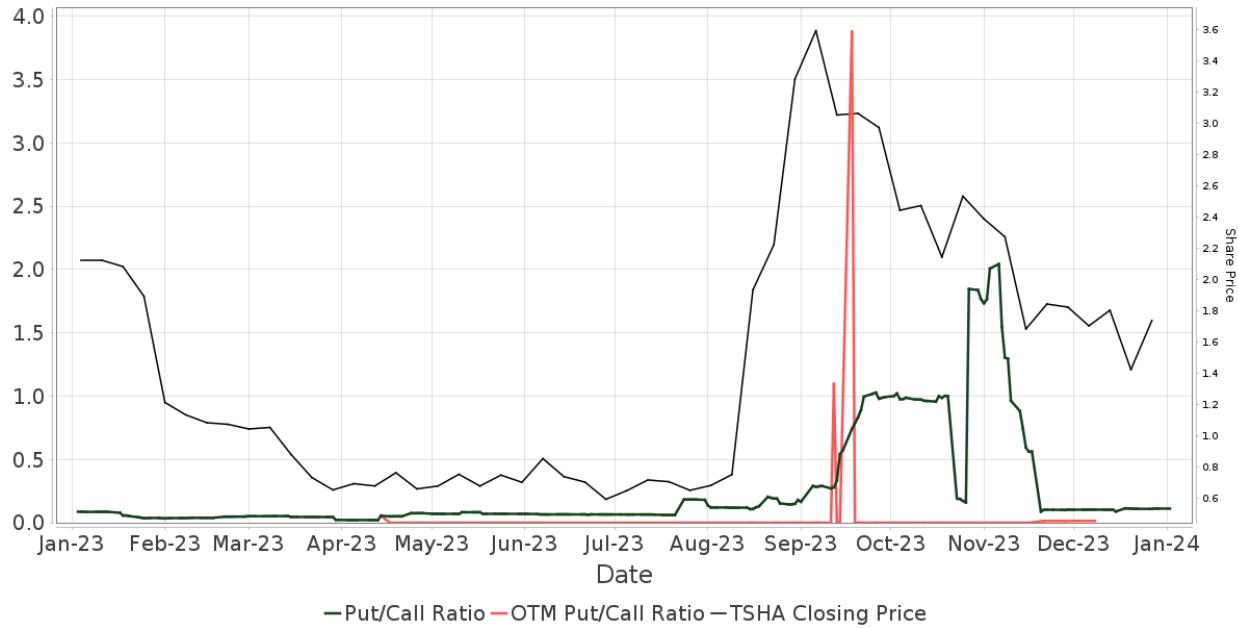
What is the Fund Sentiment?

There are [69 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 15 owner(s) or 17.86% in the last quarter. Average portfolio weight **of all**

funds dedicated to TSHA is 0.01%, a decrease of 4.40%. Total shares owned by institutions decreased in the last three months by 17.87% to 14,287K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io

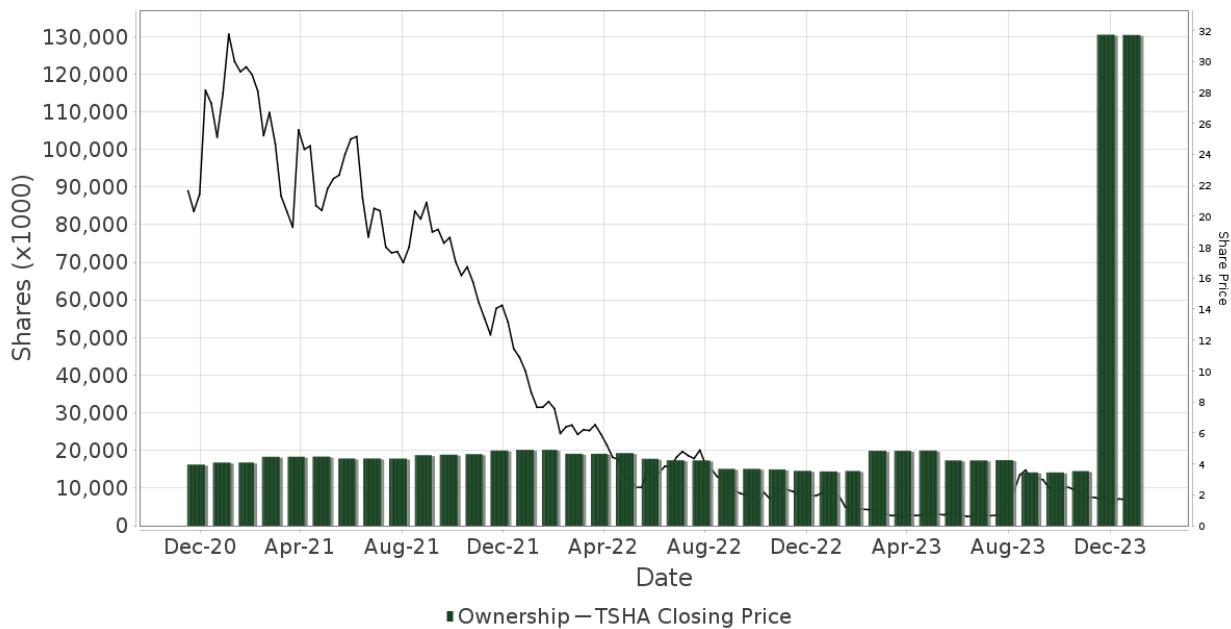


The [put/call ratio](#) of TSHA is 0.12, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 1.95% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a **decrease** of 37.67%. The firm **decreased** its portfolio allocation in TSHA by 22.73% over the last quarter.

[Laurion Capital Management](#) holds 1,172K shares representing 1.83% ownership of the company. No change in the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

JMP Securities Reiterates Taysha Gene Therapies (TSHA) Market Outperform Recommendation

August 15, 2023 — 11:19 pm EDT

Written by George Maybach for [Fintel ->](#)



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Fintel reports that on August 15, 2023, JMP Securities [reiterated](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Market Outperform** recommendation.

Analyst Price Forecast Suggests 140.18% Upside

As of August 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 140.18% from its latest reported closing price of 2.18.

See our [leaderboard of companies](#) with the largest price target upside.

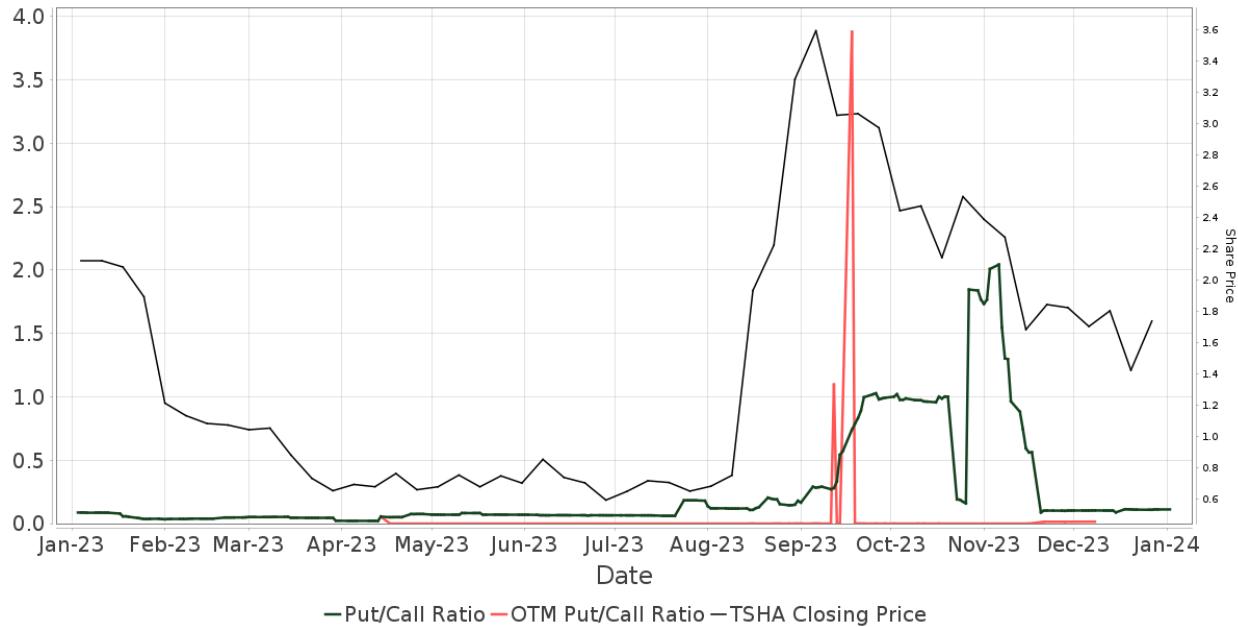
The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [69 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 15 owner(s) or 17.86% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 4.40%. Total shares owned by institutions decreased in the last three months by 17.87% to 14,287K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io



The [put/call ratio](#) of TSHA is 0.12, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 1.95% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a **decrease** of 37.67%. The firm **decreased** its portfolio allocation in TSHA by 22.73% over the last quarter.

[Laurion Capital Management](#) holds 1,172K shares representing 1.83% ownership of the company. No change in the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Taysha Gene Therapies Rally Continues

August 15, 2023 — 10:00 am EDT

Written by RTTNews.com for [RTTNews](#) ->

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(RTTNews) - Taysha Gene Therapies, Inc. (TSHA) shares are continuing a rally and gained more than 10 percent on Tuesday morning trade after the company yesterday revealed a positive Phase 1/2 trial of TSZA-102 in Rett syndrome. The clinical-stage gene therapy firm also received FDA clearance to initiate TSZA-102 in pediatric patients in the U.S.

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Further, on Monday, the company had announced a narrower net loss for the second quarter.

Currently, shares are at \$2.20, up 10 percent from the previous close of \$2.13 on a volume of 15,852,084.

Pre-Market Most Active for Aug 15, 2023 : TSZA, SQQQ, AAPL, AMD, TQQQ, SE, DASH, NVDA, AMC, NIO, APE, PLTR

August 15, 2023 — 08:28 am EDT

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The [NASDAQ 100 Pre-Market Indicator](#) is down -70.49 to 15,135.1. The total Pre-Market volume is currently 43,201,105 shares traded.

The following are the [most active stocks for the pre-market session](#):

Taysha Gene Therapies, Inc. ([TSZA](#)) is +0.3602 at \$2.49, with 4,692,055 shares traded. As reported in the last short interest update the days to cover for [TSZA](#) is 7.34132; this calculation is based on the average trading volume of the stock.

ProShares UltraPro Short QQQ ([SQQQ](#)) is +0.28 at \$19.14, with 4,348,249 shares traded. This represents a 16.85% increase from its 52 Week Low.

Apple Inc. ([AAPL](#)) is -0.71 at \$178.75, with 3,250,202 shares traded. Over the last four weeks they have had 4 up revisions for the earnings forecast, for the fiscal quarter ending Sep 2023. The consensus EPS forecast is \$1.37. As reported by Zacks, the current mean recommendation for [AAPL](#) is in the "buy range".

Advanced Micro Devices, Inc. ([AMD](#)) is -0.09 at \$111.89, with 2,306,973 shares traded. As reported by Zacks, the current mean recommendation for [AMD](#) is in the "buy range".

ProShares UltraPro QQQ ([TQQQ](#)) is -0.58 at \$39.81, with 2,287,916 shares traded. This represents a 147.27% increase from its 52 Week Low.

Sea Limited ([SE](#)) is -8.65 at \$48.25, with 1,511,801 shares traded. As reported by Zacks, the current mean recommendation for [SE](#) is in the "buy range".

DoorDash, Inc. ([DASH](#)) is -0.89 at \$79.00, with 1,392,911 shares traded. Over the last four weeks they have had 6 up revisions for the earnings forecast, for the fiscal quarter ending Sep 2023. The consensus EPS forecast is \$-0.45. [DASH's](#) current last sale is 84.95% of the target price of \$93.

NVIDIA Corporation ([NVDA](#)) is +8.87 at \$446.40, with 1,367,962 shares traded. As reported by Zacks, the current mean recommendation for [NVDA](#) is in the "buy range".

AMC Entertainment Holdings, Inc. ([AMC](#)) is +0.0202 at \$3.41, with 1,062,971 shares traded. Over the last four weeks they have had 3 up revisions for the earnings forecast, for the fiscal quarter ending Jun 2024. The consensus EPS forecast is \$-0.02. , following a 52-week high recorded in prior regular session.

NIO Inc. ([NIO](#)) is -0.26 at \$12.26, with 821,767 shares traded. [NIO's](#) current last sale is 81.73% of the target price of \$15.

AMC Entertainment Holdings, Inc. ([APE](#)) is -0.01 at \$2.06, with 588,273 shares traded.

Palantir Technologies Inc. ([PLTR](#)) is -0.12 at \$15.60, with 582,909 shares traded. Over the last four weeks they have had 3 up revisions for the earnings forecast, for the fiscal quarter ending Mar 2024. The consensus EPS forecast is \$0.03. [PLTR's](#) current last sale is 124.8% of the target price of \$12.5.

Cantor Fitzgerald Maintains Taysha Gene Therapies (TSHA) Overweight Recommendation

August 14, 2023 — 10:53 pm EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on August 14, 2023, Cantor Fitzgerald [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Overweight** recommendation.

Analyst Price Forecast Suggests 136.92% Upside

As of August 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 136.92% from its latest reported closing price of 2.21.

See our [leaderboard of companies](#) with the largest price target upside.

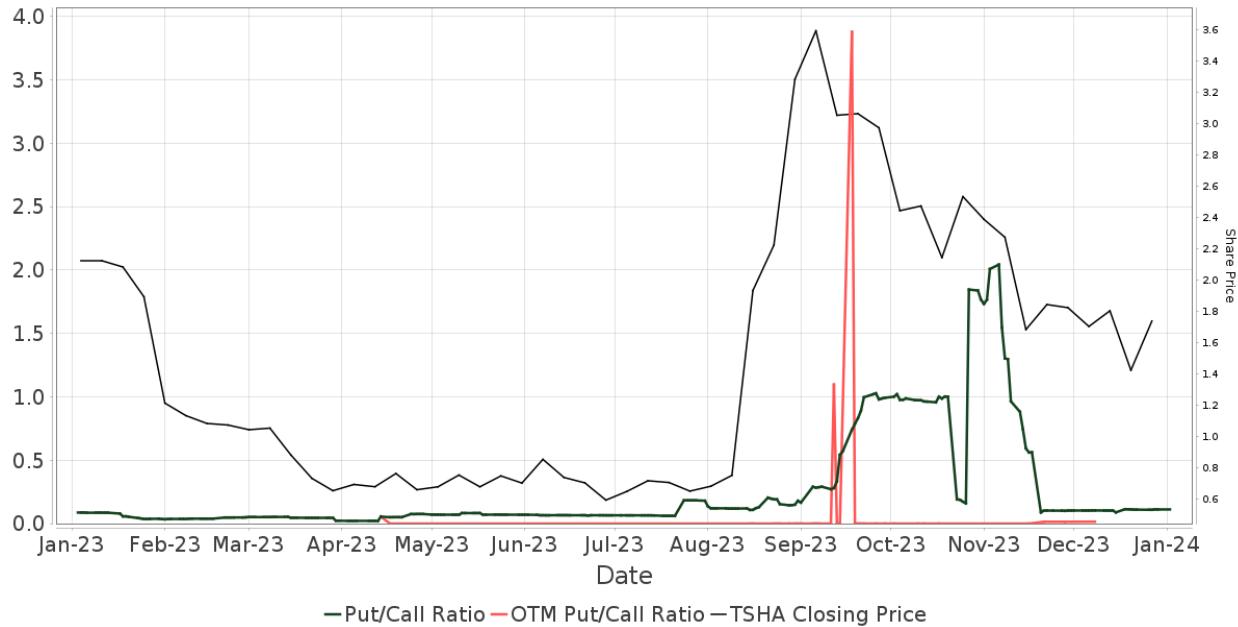
The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 93.54%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [68 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 21 owner(s) or 23.60% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 47.00%. Total shares owned by institutions decreased in the last three months by 22.66% to 14,811K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io



The [put/call ratio](#) of TSHA is 0.12, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Laurion Capital Management](#) holds 1,293K shares representing 2.01% ownership of the company. No change in the last quarter.

[Nantahala Capital Management](#) holds 1,251K shares representing 1.95% ownership of the company. In its prior filing, the firm reported owning 1,722K shares, representing a decrease of 37.67%. The firm decreased its portfolio allocation in TSHA by 22.73% over the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Health Care Sector Update for 08/14/2023: TGTX, TSHA, ZYNE, HRMY, BTAI

August 14, 2023 — 03:50 pm EDT

Written by MT Newswires for [MTNewswires](#) ->

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Health care stocks were slightly higher late Monday afternoon, with the NYSE Health Care Index adding 0.2% and the Health Care Select Sector SPDR Fund (XLV) up 0.1%.

The iShares Biotechnology ETF (IBB) was decreasing 0.2%.

In company news, TG Therapeutics ([TGTX](#)) shares jumped almost 10% after the company's chief executive officer bought about \$1.01 million in shares.

Zynerba Pharmaceuticals ([ZYNE](#)) shares surged 290% after Harmony Biosciences ([HRMY](#)) said it agreed to buy the company.

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Taysha Gene Therapies ([TSHA](#)) shares soared 165% after reporting initial data from a phase 1/2 trial of TSHA-102 and related regulatory updates.

BioXcel Therapeutics ([BTAI](#)) shares slumped 42% after the company said it plans to cut its workforce to about 80 employees from 190 as part of a strategic reprioritization.

Health Care Sector Update for 08/14/2023: TSHA, HRMY, ZYNE, BTAI

August 14, 2023 — 02:34 pm EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were rising Monday afternoon with the NYSE Health Care Index adding 0.3% and the Health Care Select Sector SPDR Fund (XLV) up 0.2%.

The iShares Biotechnology ETF (IBB) was decreasing 0.3%.

In company news, Taysha Gene Therapies ([TSHA](#)) shares more than doubled after reporting initial data from a phase 1/2 trial of TSHA-102 and related regulatory updates.

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Learn More

Zynerba Pharmaceuticals' ([ZYNE](#)) shares almost quadrupled after Harmony Biosciences ([HRMY](#)) said it agreed to buy the company.

BioXcel Therapeutics ([BTAI](#)) shares slumped 41% after the company said it plans to cut its workforce to about 80 employees from 190 as part of a strategic reprioritization.

Canaccord Genuity Reiterates Taysha Gene Therapies (TSHA) Buy Recommendation

July 06, 2023 — 06:55 pm EDT

Written by George Maybach for [Fintel](#) ->

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Fintel reports that on July 6, 2023, Canaccord Genuity [reiterated](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 711.02% Upside

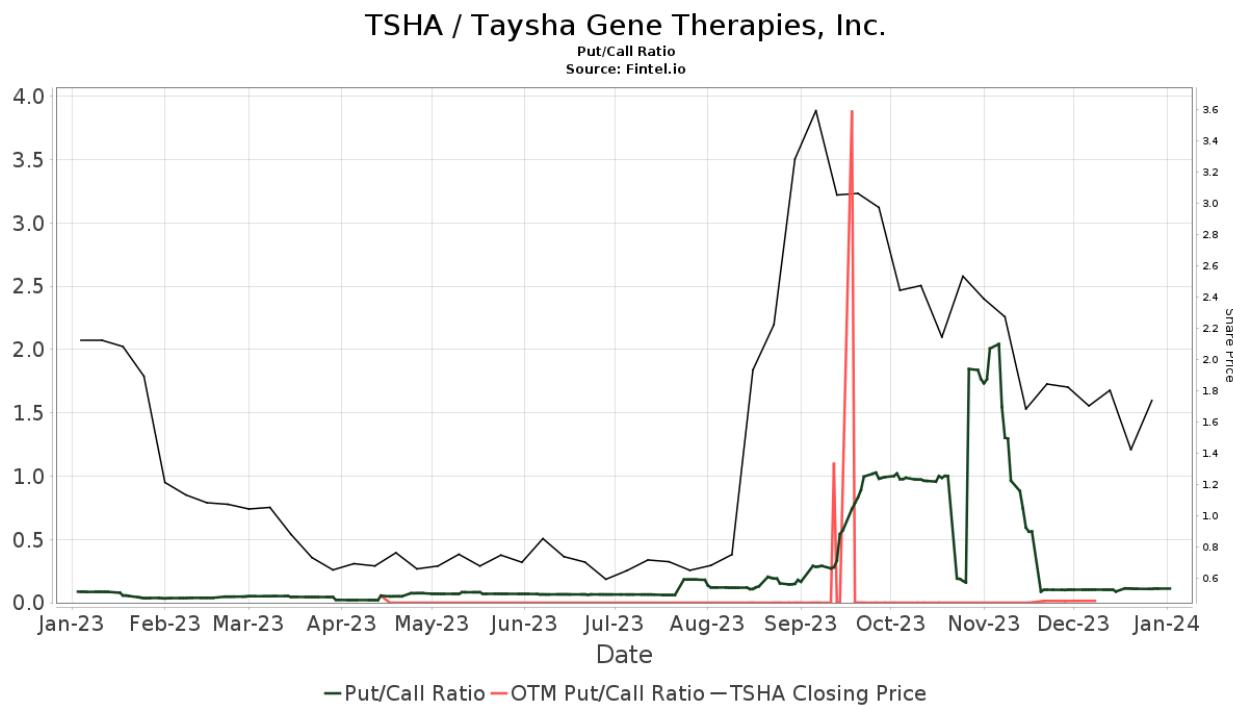
As of June 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 711.02% from its latest reported closing price of 0.65.

See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 91.39%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [78 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 15 owner(s) or 16.13% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 69.70%. Total shares owned by institutions decreased in the last three months by 12.90% to 17,341K shares.



The [put/call ratio](#) of TSHA is 0.06, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Nantahala Capital Management](#) holds 1,722K shares representing 2.68% ownership of the company. In its prior filing, the firm reported owning 3,000K shares, representing a **decrease** of 74.16%. The firm **decreased** its portfolio allocation in TSHA by 76.44% over the last quarter.

[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Laurion Capital Management](#) holds 1,293K shares representing 2.01% ownership of the company. No change in the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene

therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Chardan Capital Reiterates Taysha Gene Therapies (TSHA) Buy Recommendation

June 30, 2023 — 02:47 pm EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on June 30, 2023, Chardan Capital [reiterated](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 687.36% Upside

As of June 2, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.24. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 687.36% from its latest reported closing price of 0.66.

See our [leaderboard of companies](#) with the largest price target upside.

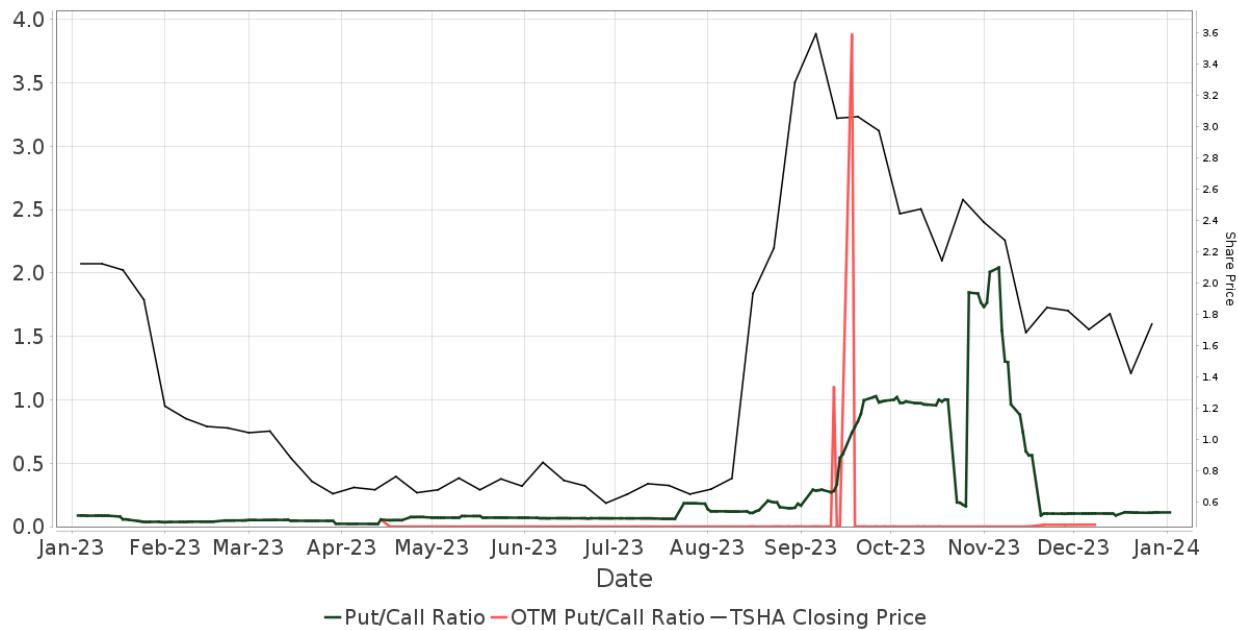
The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 91.39%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [79 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 14 owner(s) or 15.05% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 70.05%. Total shares owned by institutions decreased in the last three months by 12.87% to 17,346K shares.

TSHA / Taysha Gene Therapies, Inc.

Put/Call Ratio
Source: Fintel.io

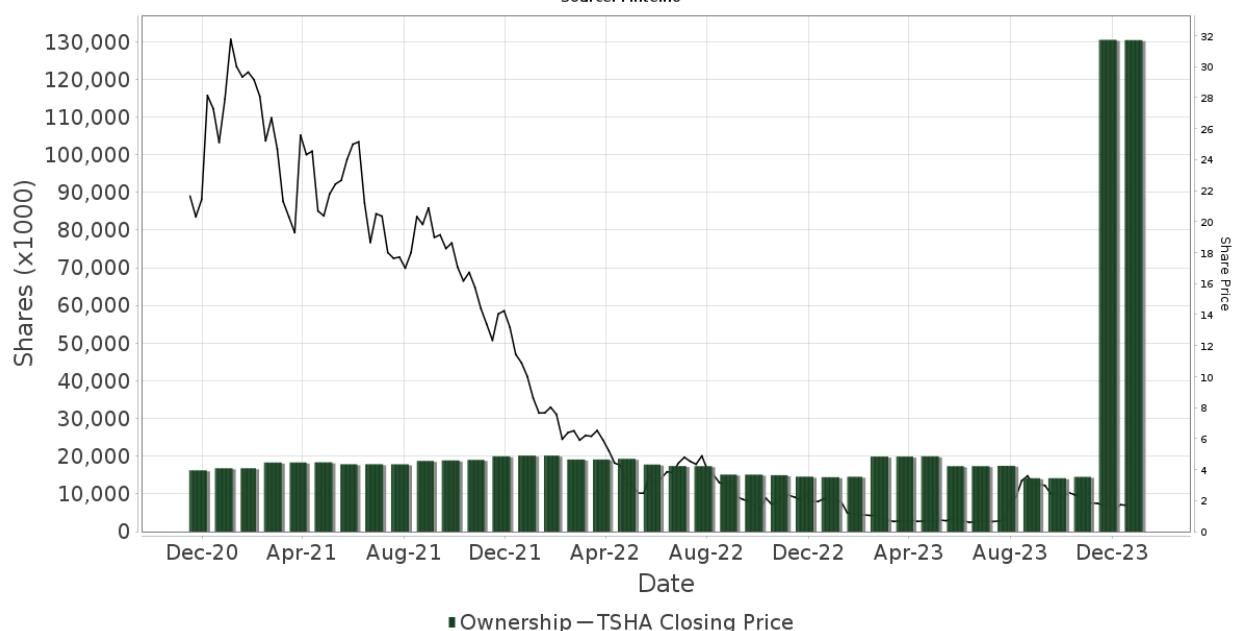


The [put/call ratio](#) of TSHA is 0.07, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Nantahala Capital Management](#) holds 1,722K shares representing 2.68% ownership of the company. In its prior filing, the firm reported owning 3,000K shares, representing a

decrease of 74.16%. The firm **decreased** its portfolio allocation in TSJA by 76.44% over the last quarter.

[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Laurion Capital Management](#) holds 1,293K shares representing 2.01% ownership of the company. No change in the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information *(This description is provided by the company.)*

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Truist Securities Maintains Taysha Gene Therapies (TSJA) Buy Recommendation

May 16, 2023 — 08:14 am EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on May 15, 2023, Truist Securities [maintained](#) coverage of Taysha Gene Therapies ([NASDAQ:TSHA](#)) with a **Buy** recommendation.

Analyst Price Forecast Suggests 671.79% Upside

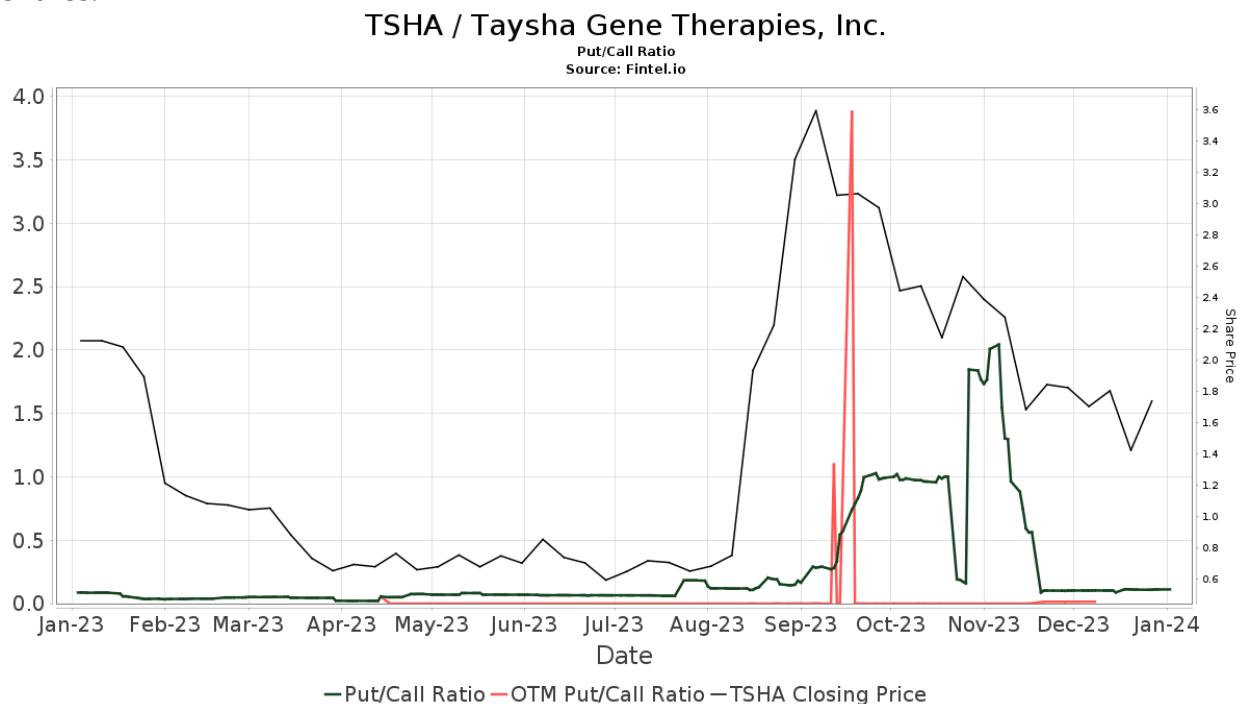
As of May 11, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.35. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 671.79% from its latest reported closing price of 0.69.

See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 91.39%. The projected annual non-GAAP [EPS](#) is -1.97.

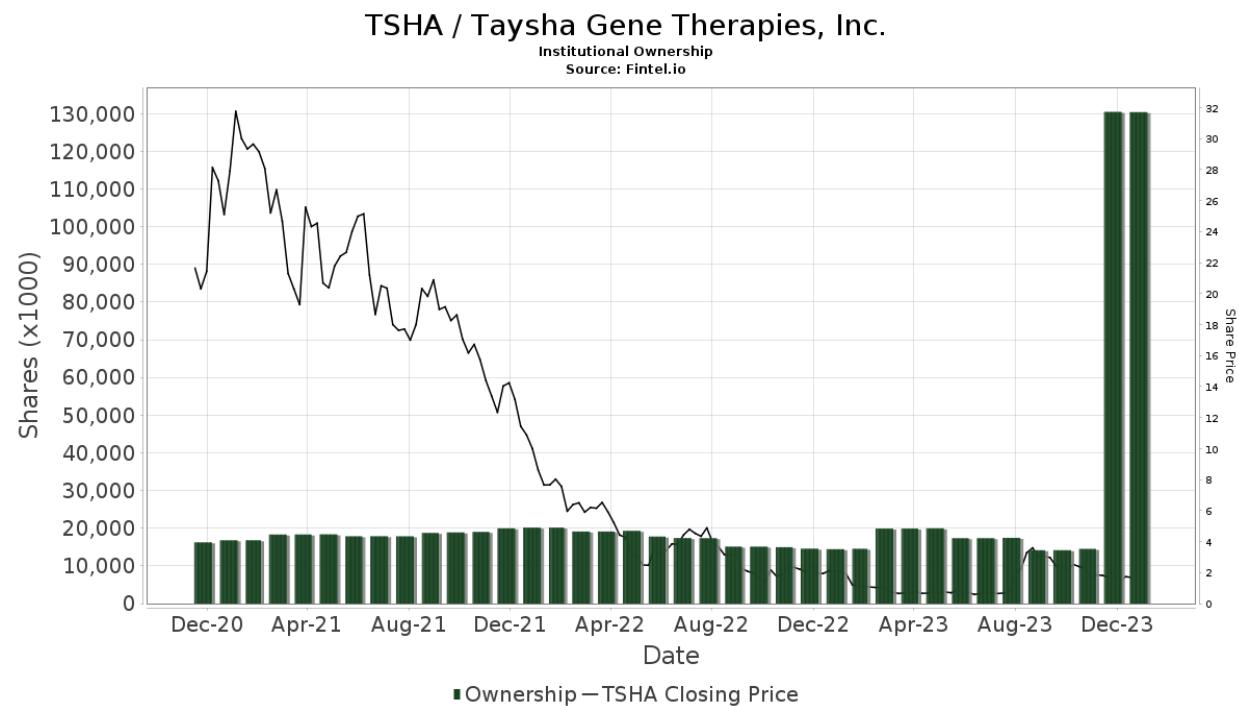
What is the Fund Sentiment?

There are [84 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 8 owner(s) or 8.70% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.01%, a decrease of 50.59%. Total shares owned by institutions decreased in the last three months by 12.63% to 17,395K shares.



The [put/call ratio](#) of TSHA is 0.08, indicating a bullish outlook.

What are Other Shareholders Doing?



[Nantahala Capital Management](#) holds 1,722K shares representing 2.68% ownership of the company. In its prior filing, the firm reported owning 3,000K shares, representing a **decrease** of 74.16%. The firm **decreased** its portfolio allocation in TSHA by 76.44% over the last quarter.

[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. No change in the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. No change in the last quarter.

[Laurion Capital Management](#) holds 1,293K shares representing 2.01% ownership of the company. No change in the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. In its prior filing, the firm reported owning 729K shares, representing an **increase** of 37.02%. The firm **increased** its portfolio allocation in TSHA by 71.70% over the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Needham Reiterates Taysha Gene Therapies (TSHA) Buy Recommendation

May 12, 2023 — 07:54 am EDT

Written by George Maybach for [Fintel ->](#)

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Fintel reports that on May 12, 2023, Needham [reiterated](#) coverage of Taysha Gene Therapies (NASDAQ:TSHA) with a **Buy** recommendation.

Analyst Price Forecast Suggests 654.48% Upside

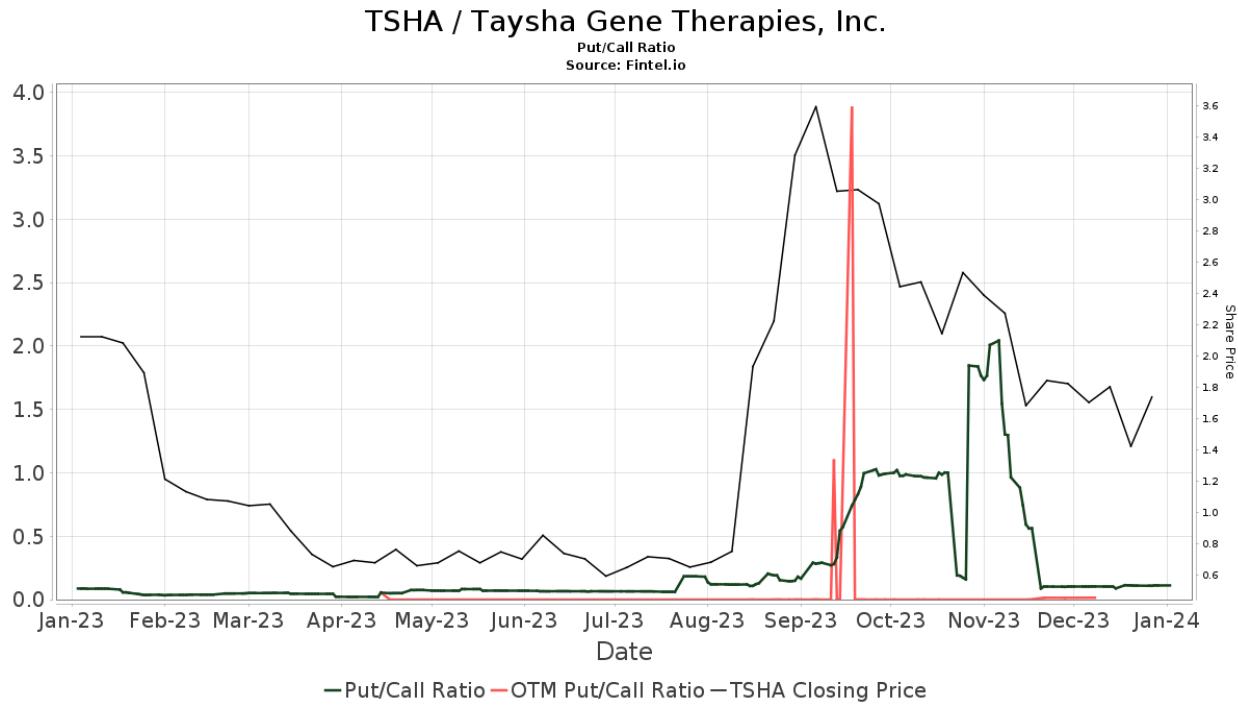
As of May 11, 2023, the average one-year [price target](#) for Taysha Gene Therapies is 5.35. The forecasts range from a low of 0.71 to a high of \$13.65. The average price target represents an increase of 654.48% from its latest reported closing price of 0.71.

See our [leaderboard of companies](#) with the largest price target upside.

The projected annual revenue for Taysha Gene Therapies is 1MM, a decrease of 75.21%. The projected annual non-GAAP [EPS](#) is -1.97.

What is the Fund Sentiment?

There are [89 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 6 owner(s) or 6.32% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.02%, an increase of 67.66%. Total shares owned by institutions increased in the last three months by 29.13% to 19,875K shares.

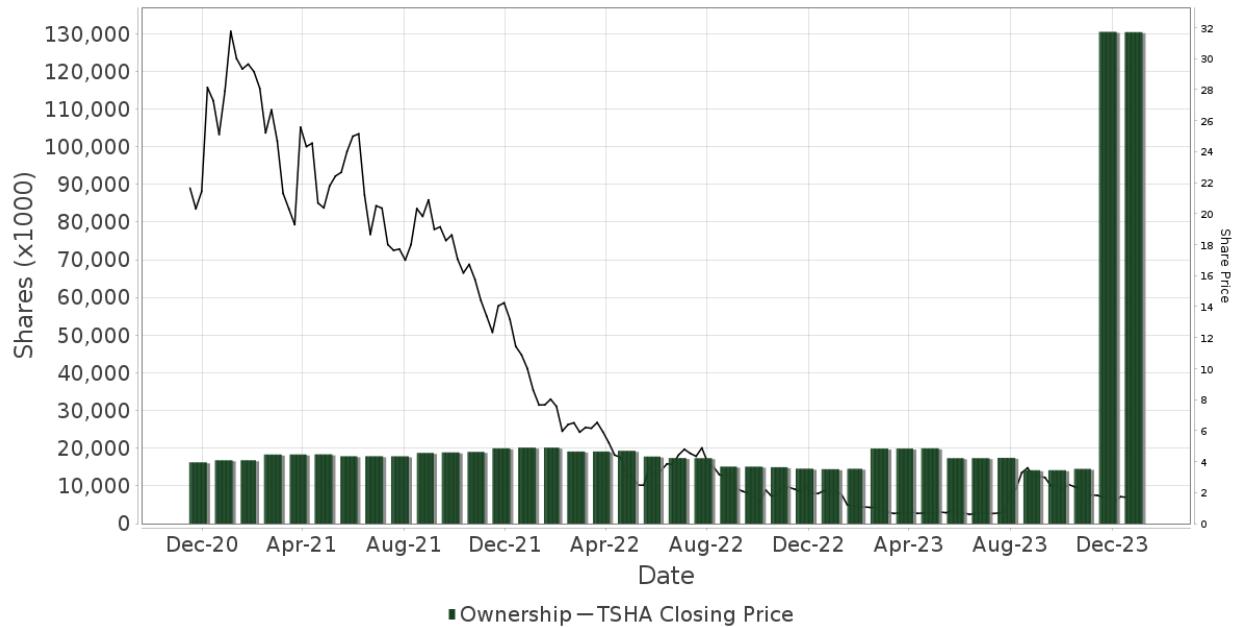


The [put/call ratio](#) of TSHA is 0.08, indicating a bullish outlook.

What are Other Shareholders Doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Nantahala Capital Management](#) holds 3,000K shares representing 4.67% ownership of the company. In its prior filing, the firm reported owning 1,611K shares, representing **an increase** of 46.30%. The firm **increased** its portfolio allocation in TSHA by 109.18% over the last quarter.

[Tybourne Capital Management](#) holds 1,500K shares representing 2.34% ownership of the company. In its prior filing, the firm reported owning 500K shares, representing **an increase** of 66.67%. The firm **increased** its portfolio allocation in TSHA by 264.23% over the last quarter.

[Artal Group](#) holds 1,441K shares representing 2.25% ownership of the company. In its prior filing, the firm reported owning 1,041K shares, representing **an increase** of 27.76%. The firm **increased** its portfolio allocation in TSHA by 111.97% over the last quarter.

[Laurion Capital Management](#) holds 1,293K shares representing 2.01% ownership of the company. In its prior filing, the firm reported owning 443K shares, representing **an increase** of 65.76%. The firm **increased** its portfolio allocation in TSHA by 171.03% over the last quarter.

[VTSMX - Vanguard Total Stock Market Index Fund Investor Shares](#) holds 1,158K shares representing 1.80% ownership of the company. In its prior filing, the firm reported owning 729K shares, representing **an increase** of 37.02%. The firm **increased** its portfolio allocation in TSHA by 71.70% over the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

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Prefabricated Cabins

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Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

See all Taysha Gene Therapies [regulatory filings](#).

Fmr Cuts Stake in Taysha Gene Therapies (TSHA)

February 09, 2023 — 12:43 pm EST

Written by Fintel Staff for [Fintel](#) ->

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Fintel reports that [Fmr](#) has filed a [13G/A form](#) with the SEC disclosing ownership of 3.20MM shares of Taysha Gene Therapies Inc ([TSHA](#)). This represents 5.132% of the company.

In their previous filing dated November 10, 2022 they reported 3.27MM shares and 5.95% of the company, **a decrease in shares of 2.13% and a decrease in total ownership of 0.82%** (calculated as current - previous percent ownership).

Analyst Price Forecast Suggests 1,376.48% Upside

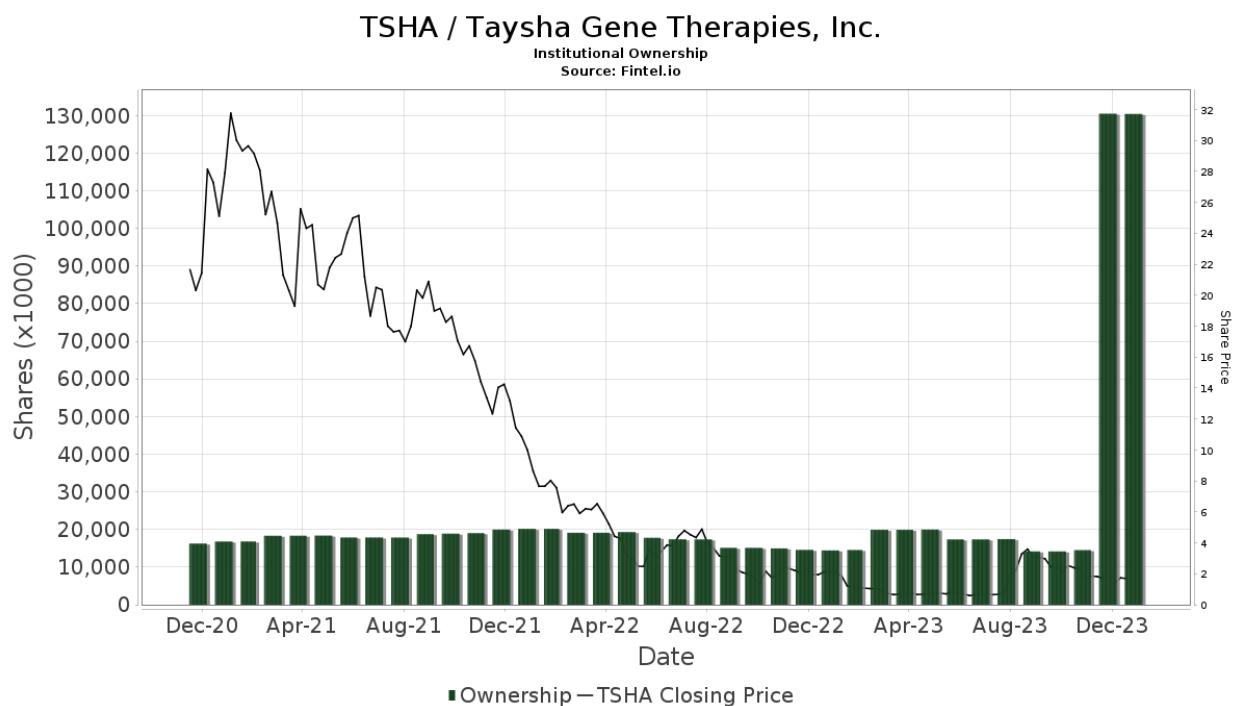
As of February 8, 2023, the average one-year [price target](#) for Taysha Gene Therapies is \$16.68. The forecasts range from a low of \$3.03 to a high of \$25.20. The average price target represents an increase of 1,376.48% from its latest reported closing price of \$1.13.

The projected annual revenue for Taysha Gene Therapies is \$1MM. The projected annual [EPS](#) is -\$1.97.

What is the Fund Sentiment?

There are [96 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 88 owner(s) or 47.83% in the last quarter. Average portfolio weight **of all funds** dedicated to TSHA is 0.02%, an increase of 20.43%. Total shares owned by institutions decreased in the last three months by 1.29% to 14,786K shares. The [put/call ratio](#) of TSHA is 0.04, indicating a bullish outlook.

What are large shareholders doing?



[Nantahala Capital Management](#) holds 1,611K shares representing 2.58% ownership of the company. In it's prior filing, the firm reported owning 1,643K shares, representing a

decrease of 2.02%. The firm **decreased** its portfolio allocation in TSHA by 31.48% over the last quarter.

[Artal Group](#) holds 1,041K shares representing 1.67% ownership of the company. No change in the last quarter.

[FDGRX - Fidelity Growth Company Fund](#) holds 920K shares representing 1.47% ownership of the company. In it's prior filing, the firm reported owning 951K shares, representing a **decrease** of 3.44%. The firm **decreased** its portfolio allocation in TSHA by 30.96% over the last quarter.

[FIBOX - Biotechnology Portfolio](#) holds 812K shares representing 1.30% ownership of the company. In it's prior filing, the firm reported owning 883K shares, representing a **decrease** of 8.77%. The firm **decreased** its portfolio allocation in TSHA by 37.25% over the last quarter.

[Franklin Resources](#) holds 811K shares representing 1.30% ownership of the company. No change in the last quarter.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

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Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

BTIG Downgrades Taysha Gene Therapies (TSHA)

February 03, 2023 — 07:36 am EST

Written by Fintel Staff for [Fintel](#) ->

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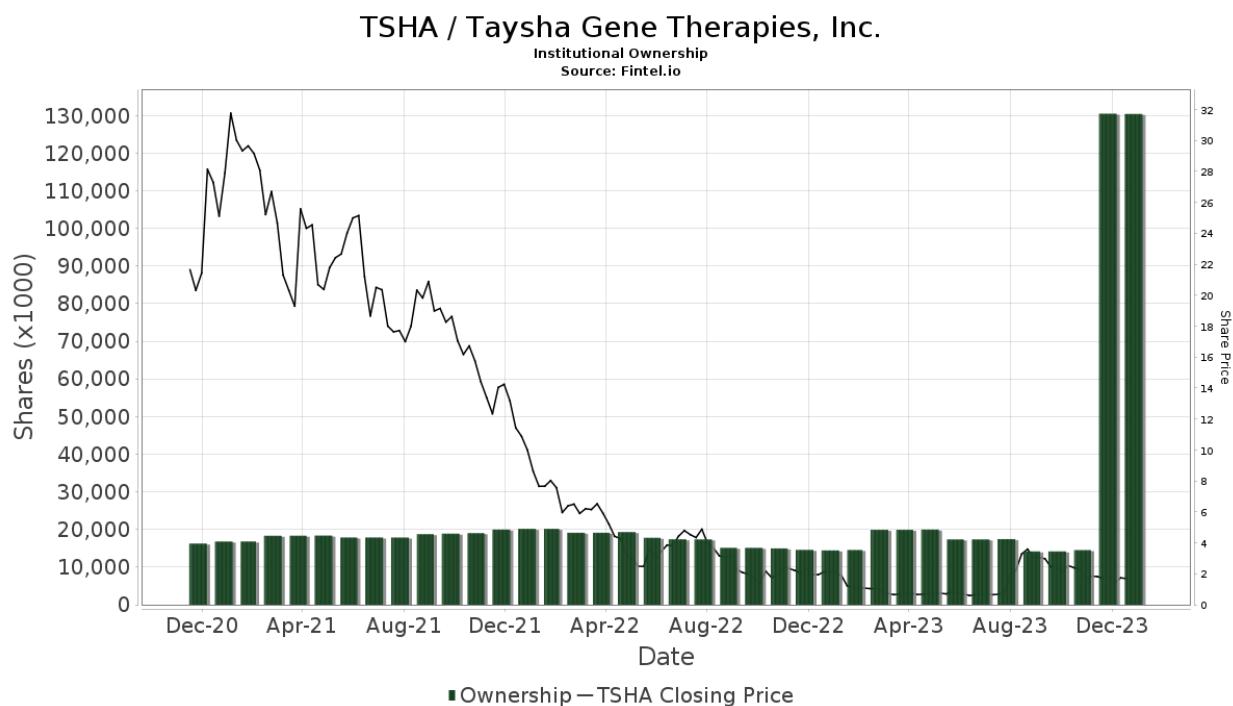
On February 2, 2023, BTIG downgraded their [outlook](#) for Taysha Gene Therapies from **Buy to Neutral**.

Analyst Price Forecast Suggests 1,278.86% Upside

As of February 3, 2023, the average one-year [price target](#) for Taysha Gene Therapies is \$16.68. The forecasts range from a low of \$3.03 to a high of \$25.20. The average price target represents an increase of 1,278.86% from its latest reported closing price of \$1.21.

The projected annual revenue for Taysha Gene Therapies is \$1MM. The projected annual [EPS](#) is \$-1.97.

What are large shareholders doing?



[Nantahala Capital Management](#) holds 1,610,822 shares representing 2.58% ownership of the company. In it's prior filing, the firm reported owning 1,643,438 shares,

representing a **decrease** of 2.02%. The firm **decreased** its portfolio allocation in TSHA by 31.48% over the last quarter.

[Artal Group](#) holds 1,040,882 shares representing 1.67% ownership of the company. No change in the last quarter.

[FDGRX - Fidelity Growth Company Fund](#) holds 919,727 shares representing 1.47% ownership of the company. In it's prior filing, the firm reported owning 951,337 shares, representing a **decrease** of 3.44%. The firm **decreased** its portfolio allocation in TSHA by 30.96% over the last quarter.

[FIBOX - Biotechnology Portfolio](#) holds 811,647 shares representing 1.30% ownership of the company. In it's prior filing, the firm reported owning 882,847 shares, representing a **decrease** of 8.77%. The firm **decreased** its portfolio allocation in TSHA by 37.25% over the last quarter.

[Franklin Resources](#) holds 811,069 shares representing 1.30% ownership of the company. No change in the last quarter.

Fund Sentiment

There are [102 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 80 owner(s) or 43.96%.

Average portfolio weight **of all funds** dedicated to US:TSHA is 0.0195%, an increase of 15.3897%. Total shares owned by institutions decreased in the last three months by 2.90% to 14,534K shares.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

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Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives.

Jefferies Downgrades Taysha Gene Therapies (TSHA)

February 01, 2023 — 06:41 pm EST

Written by Fintel Staff for [Fintel](#) ->

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On February 1, 2023, Jefferies downgraded their [outlook](#) for Taysha Gene Therapies from **Buy** to **Hold**.

Analyst Price Forecast Suggests 917.33% Upside

As of February 1, 2023, the average one-year [price target](#) for Taysha Gene Therapies is \$16.68. The forecasts range from a low of \$3.03 to a high of \$25.20. The average price target represents an increase of 917.33% from its latest reported closing price of \$1.64.

The projected annual revenue for Taysha Gene Therapies is \$1MM. The projected annual [EPS](#) is \$-1.97.

What are large shareholders doing?

TSHA / Taysha Gene Therapies, Inc.

Institutional Ownership
Source: Fintel.io



[Nantahala Capital Management](#) holds 1,610,822 shares representing 2.58% ownership of the company. In its prior filing, the firm reported owning 1,643,438 shares, representing a **decrease** of 2.02%. The firm **decreased** its portfolio allocation in TSHA by 31.48% over the last quarter.

[Artal Group](#) holds 1,040,882 shares representing 1.67% ownership of the company. No change in the last quarter.

[FDGRX - Fidelity Growth Company Fund](#) holds 919,727 shares representing 1.47% ownership of the company. In its prior filing, the firm reported owning 951,337 shares, representing a **decrease** of 3.44%. The firm **decreased** its portfolio allocation in TSHA by 30.96% over the last quarter.

[FIBOX - Biotechnology Portfolio](#) holds 811,647 shares representing 1.30% ownership of the company. In its prior filing, the firm reported owning 882,847 shares, representing a **decrease** of 8.77%. The firm **decreased** its portfolio allocation in TSHA by 37.25% over the last quarter.

[Franklin Resources](#) holds 811,069 shares representing 1.30% ownership of the company. No change in the last quarter.

Fund Sentiment

There are [100 funds or institutions reporting positions](#) in Taysha Gene Therapies. This is a decrease of 82 owner(s) or 45.05%.

Average portfolio weight **of all funds** dedicated to US:TSHA is 0.0187%, an increase of 8.3676%. Total shares owned by institutions decreased in the last three months by 2.56% to 14,517K shares.

Taysha Gene Therapies Background Information

(This description is provided by the company.)

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Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate our treatments from bench to bedside. Taysha Gene Therapies has combined its team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha Gene Therapies leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives

These 2 Stocks May Be Reaching a Bottom; Insiders Are Buying the Dip

November 03, 2022 — 08:26 pm EDT

Written by Marty Shtrubel for [TipRanks ->](#)

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One thing investors are not lacking for after 2022's market rout: beaten-down stocks going for cheap compared to levels seen at the start of the year. The problem is how

can investors sift through the stock debris to pick out the names which will dust themselves down and push ahead again?

As with anything, there are multiple ways to run a stock through the litmus test, but one tried-and-true method is to watch out for the moves the insiders make. These corporate officers know the inner workings of the companies they serve like no one else and when those running the ship load up on shares, it sends a signal they must believe they are undervalued. To keep the playing field level, they must make their purchases (or sells for that matter) public and investors can track the transactions.

The TipRanks [Insiders' Hot Stocks](#) tool provides just such information and we've dug up the details on two names which fit a specific profile; down by significant amounts this year but which those in the know have been getting the wallet out for recently, signifying the possibility a bottom might be in sight. Even better, Street analysts also see the pair as good bets; both are rated as Strong Buys by the analyst consensus and projected to deliver strong gains over the coming months. Let see what all the fuss is about.

Insmed Incorporated ([INSM](#))

We'll start in the biotech sector with Insmed, a company concentrating on the development of treatments for rare diseases.

All biotechs hope to get a drug across the finish line and out to the public and this is a feat already achieved by Insmed. The company's Arikayce received the FDA's nod of approval in 2018 and is indicated to treat refractory nontuberculous mycobacteria (NTM) lung disease caused by *Mycobacterium avium* complex (MAC) infection.

In the recently released Q3 report, Arikayce's sales increased by 45% year-over-year to reach \$67.7 million. This keeps the company on track to meet its 2022 target of at least 30% sales growth.

In contrast to many biotech company, then, Insmed has a reliable revenue generator and that can help with the progress of the pipeline. Drugs currently being developed include brensocatib, which is earmarked as a treatment for patients with bronchiectasis, with enrollment currently taking place for the Phase 3 ASPEN study. Enrollment should be completed in 1Q23, with a topline data readout anticipated in 2Q24.

Brensocatib is also being tested in a Phase 2 pharmacokinetic/pharmacodynamic trial in cystic fibrosis (CF) patients, including both those who are and are not currently taking background CF transmembrane conductance regulator (CFTR) modulator medications.

This study is completely enrolled and there should be topline data released for both groups of patients by the end of 2022.

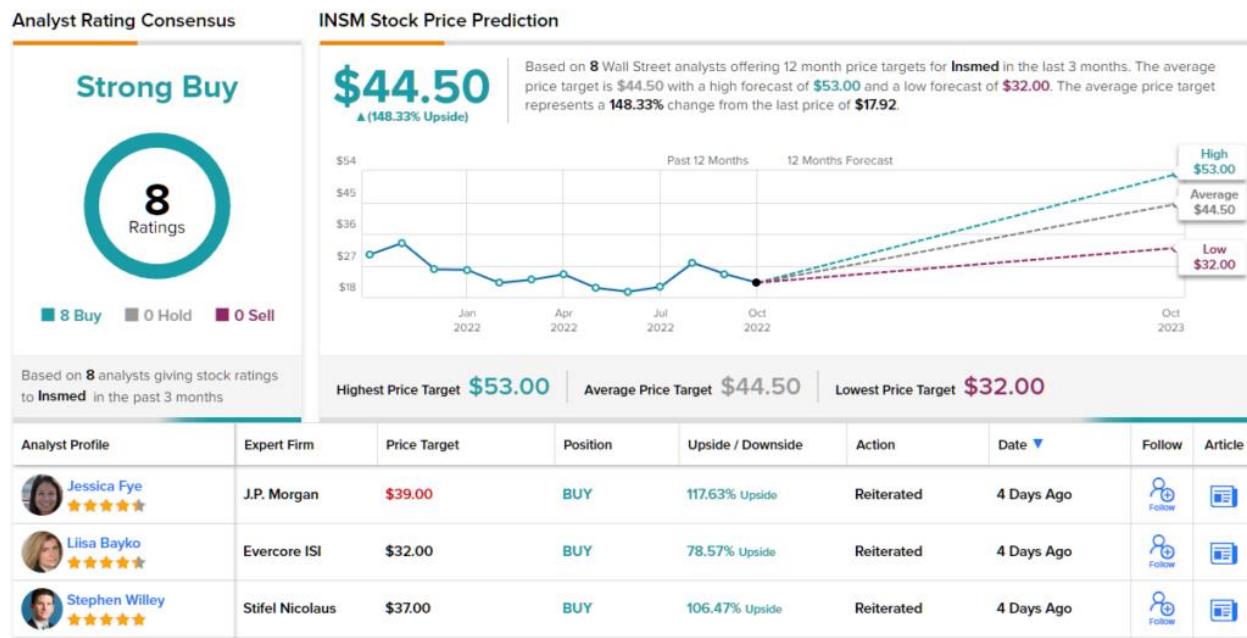
The company is also enrolling for two Phase 2 studies of treprostinil palmitil inhalation powder (TPIP), while there is also a post-marketing confirmatory, frontline clinical trial program of Arikayce in progress (the ARISE and ENCORE trials). Data from the former trial should be put forth throughout 2023.

Despite the sound pipeline, INSM shares are down 34% for the year. Yet, two insiders evidently think the time is right to pounce. This week, director Melvin Sharoky bought 30,000 shares now worth 527,700, while director Leo Lee purchased 45,000 shares worth \$791,550.

Stifel's analyst [Stephen Willey](#) is also impressed with INSM's execution, writing: "Operationally, we believe continued growth of the U.S. Arikayce business remains encouraging – particularly in the context of continued COVID/Fx headwinds in Japan. Clinically, we believe YE22 results from the P2 trial evaluating brensocatib in CF and preliminary 1H23 PRO data from ARISE could prove interesting (NSP inhibition) and important (ENCORE visibility) events, respectively."

Willey's confidence is conveyed with a Buy rating and \$37 price target, suggesting shares have room for robust growth of 106% in the year ahead. (To watch Willey's track record, [click here](#))

Willey's bullish thesis gets the full backing of his colleagues. The stock gets a full house of Buys – 8, in total – which all coalesce to a Strong Buy consensus rating. The average target is even more bullish than Willey will allow; at \$44.50, the figure represents upside of ~148% from current levels. ([See Insmed stock forecast on TipRanks](#))



Taysha Gene Therapies ([TSHA](#))

For one biotech to another. Taysha Gene Therapies' modus operandi is to develop and bring to market a variety of gene therapies to treat monogenic central nervous system (CNS) diseases – both common and rare.

At the forefront of its AAV (adeno-associated virus) gene therapy pipeline, is the most advanced clinical asset, TSHA-120, indicated to treat giant axonal neuropathy (GAN), a rare inherited genetic condition that impacts both the central and peripheral nervous systems.

TSHA-120 has received orphan drug and rare pediatric disease designations from the FDA and data from the phase 1/2 clinical trial showed that individuals with giant axonal neuropathy (GAN) who took the treatment reclaimed sensory nerve amplitudes (SNAPs) whilst also displaying clinical improvement. The company anticipates meeting with the FDA on December 13th for an end-of-Phase 2 Type B meeting in which the regulatory path forward for the drug will be discussed.

The pipeline also boasts TSHA-102, the first-and-only gene therapy being developed to treat Rett syndrome, rare genetic condition that affects the development of the brain. TSHA-102 has obtained orphan drug and rare pediatric disease designations from the FDA and data from the Phase 1/2 study is expected in 1H23.

Drug development is a costly venture and biotechs need money to progress its pipeline. Here, the company had a positive development recently. In October, Taysha announced that Astellas is making a \$50 million strategic investment in the company, which gives it

a ~15% stake along with the option to secure exclusive licenses for TSHA-102 for Rett syndrome and TSHA-120 for giant axonal neuropathy (GAN).

It's not the only investment made in recent times. This week, director Paul B Manning loaded up - by buying 1.5 million shares, for a total of \$3 million. The purchase comes at a time of a massive pullback for the stock, which is now down 83% on a year-to-date basis.

Looking at Astellas' investment, and given its track record, Baird analyst [Jack Allen](#) thinks it signals some serious intent.

"While we note this deal may ultimately result in Taysha ceding control of its two lead programs, we are quite encouraged by the interest being expressed by Astellas and note that Astellas' clear interest in the gene therapy space could potentially lead to a takeout offer in the near/medium-term should TSHA-120 and TSHA-102 produce positive updates in 1H23," Allen opined.

To this end, Allen rates the shares an Outperform (i.e., Buy) while his \$21 price target makes room for a whooping 977% in the year ahead. (To watch Allen's track record, [click here](#))

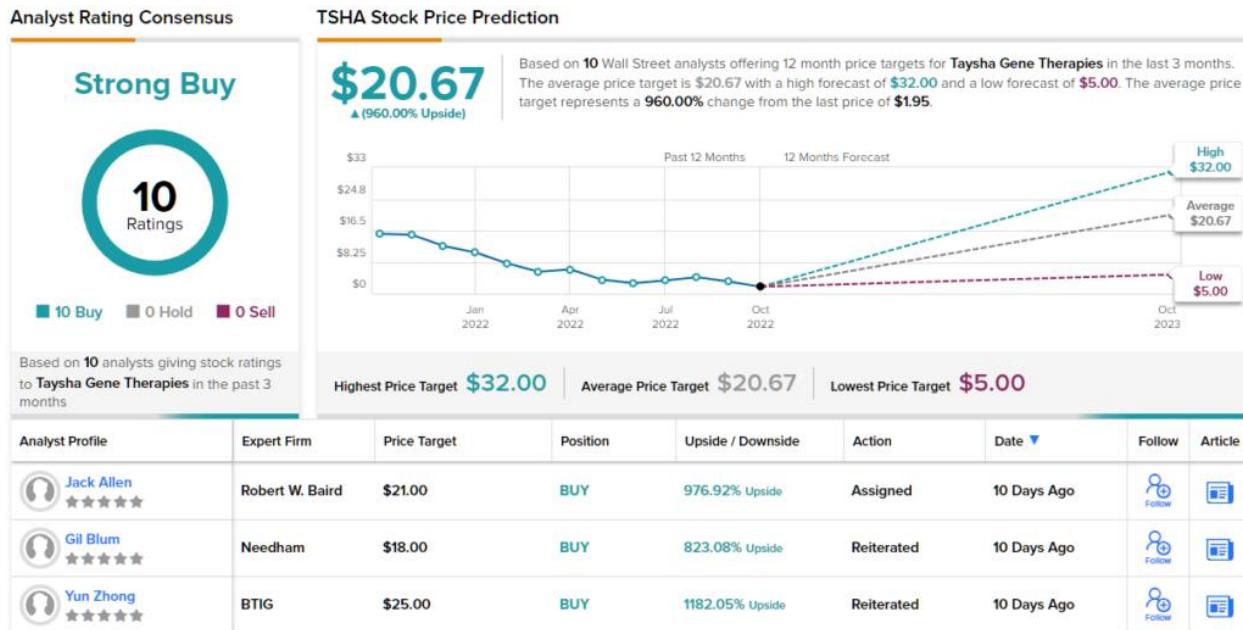
Think that is some outlandish forecast? Well, other analysts are hardly less enthusiastic. The Street's average target stands at \$20.67, which could generate returns of 960% over the coming months. The ratings are no less effusive; with a unanimous set of 10 Buys, the stock receives a Strong Buy consensus rating. ([See TSHA stock forecast on TipRanks](#))

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Prefabricated Cabins

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To find good ideas for stocks trading at attractive valuations, visit TipRanks' [Best Stocks to Buy](#), a newly launched tool that unites all of TipRanks' equity insights.

Disclaimer: The opinions expressed in this article are solely those of the featured analysts. The content is intended to be used for informational purposes only. It is very important to do your own analysis before making any investment.

Paul Manning increases stake in Taysha Gene Therapies Inc (TSHA)

November 02, 2022 — 07:28 pm EDT

Written by Fintel Staff for [Fintel](#) ->

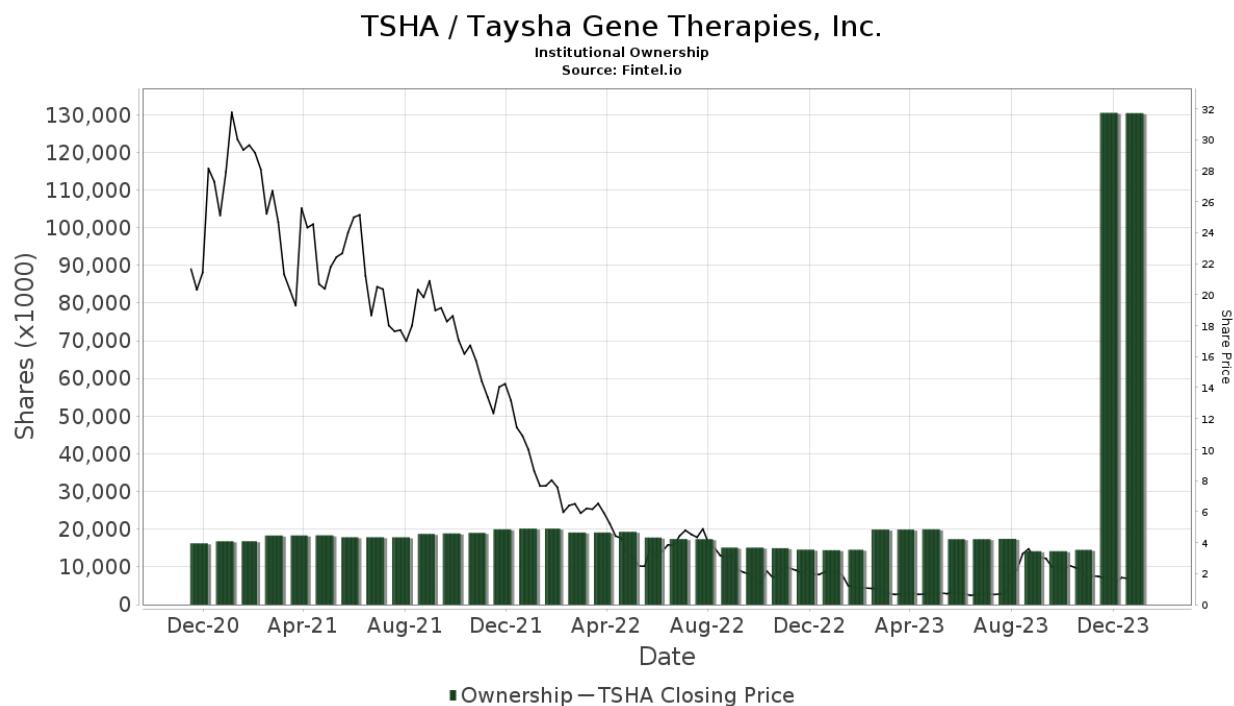
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[Manning Paul B](#) has filed a [13D/A form](#) with the SEC disclosing ownership of 6,989,861 shares of Taysha Gene Therapies Inc ([TSHA](#)). This represents 11.2% of the company.

In their previous filing dated October 5, 2020 they reported 5,295,307 shares and 14.30% of the company, an increase in shares of 32.00% and a decrease in total ownership of 3.10% (calculated as current - previous percent ownership).

They have filed 2 13D/G filings since October 5, 2020.

What are other large shareholders doing?



[Fmr Llc](#) holds 3,295,689 shares representing 5.28% ownership of the company. In it's prior filing, the firm reported owning 3,294,821 shares, representing **an increase** of 0.03%. The firm decreased its portfolio allocation in TSHA by 28.83% over the last quarter.

[Nantahala Capital Management, LLC](#) holds 1,643,438 shares representing 2.64% ownership of the company. In it's prior filing, the firm reported owning 1,333,947 shares, representing **an increase** of 18.83%. The firm decreased its portfolio allocation in TSHA by 20.32% over the last quarter.

[Vanguard Group Inc](#) holds 1,070,802 shares representing 1.72% ownership of the company. In it's prior filing, the firm reported owning 1,012,164 shares, representing **an increase** of 5.48%. The firm decreased its portfolio allocation in TSHA by 28.27% over the last quarter.

[BlackRock Inc.](#) holds 1,046,490 shares representing 1.68% ownership of the company. In its prior filing, the firm reported owning 2,193,703 shares, representing a **decrease** of 109.62%. The firm decreased its portfolio allocation in TSHA by 67.57% over the last quarter.

[Artal Group S.A.](#) holds 1,040,882 shares representing 1.67% ownership of the company. No change in the last quarter.

What is the overall institutional sentiment?

There are [182 funds or institutions reporting positions](#) in Taysha Gene Therapies Inc. This is a decrease of 16 owner(s) or 8.08%.

Average portfolio weight of *all funds* dedicated to Taysha Gene Therapies Inc is 0.0169%, a decrease of 16.5226%. Total shares owned by institutions decreased in the last three months by 13.72% to 14,968,777 shares.

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Based on this information, institutional sentiment is bearish.

Great week for Taysha Gene Therapies, Inc. (NASDAQ:TSHA) institutional investors after losing 82% over the previous year

October 26, 2022 — 06:00 am EDT

Written by Simply Wall St for [Simply Wall St ->](#)

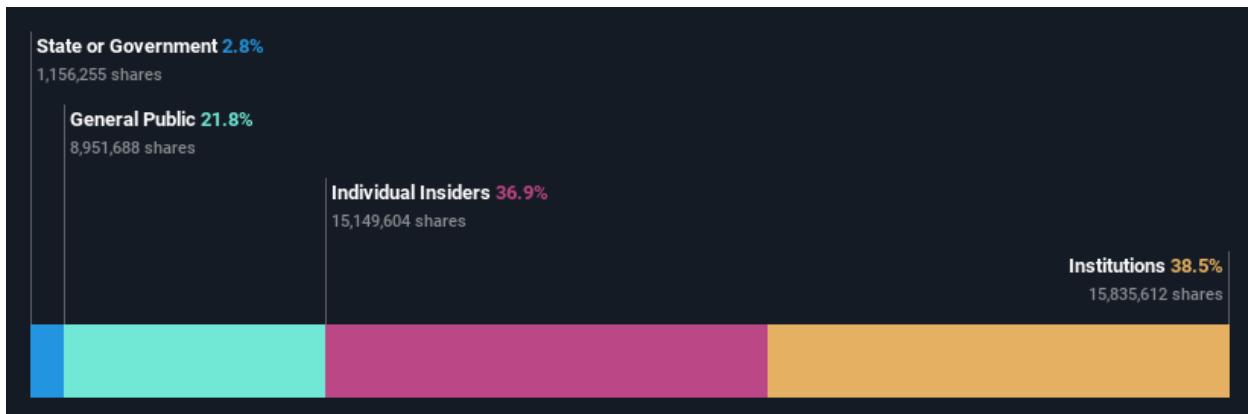
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If you want to know who really controls Taysha Gene Therapies, Inc. (NASDAQ:TSHA), then you'll have to look at the makeup of its share registry. With 39% stake, institutions possess the maximum shares in the company. In other words, the group stands to gain the most (or lose the most) from their investment into the company.

Institutional investors would probably welcome last week's 97% increase in share prices after a year of 82% losses as a sign that returns are likely to begin trending higher.

In the chart below, we zoom in on the different ownership groups of Taysha Gene Therapies.



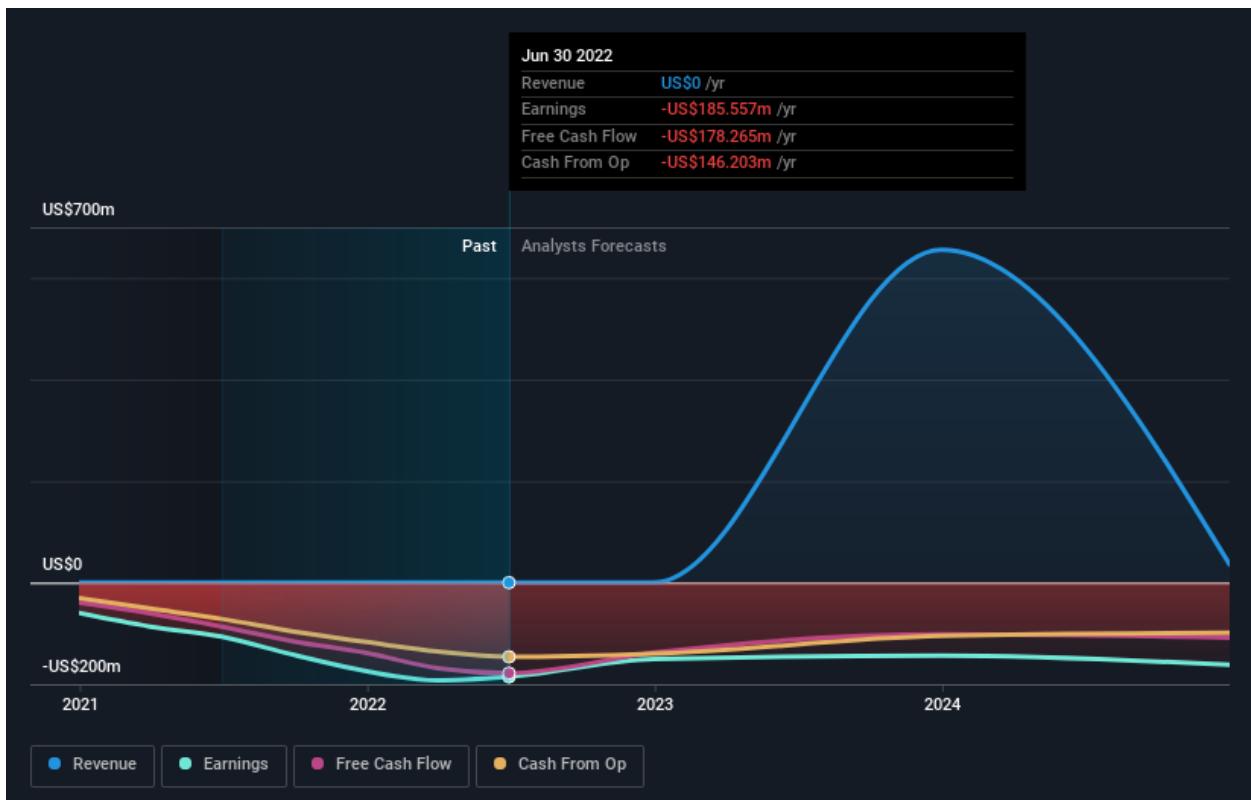
NasdaqGS:TSHA Ownership Breakdown October 26th 2022

What Does The Institutional Ownership Tell Us About Taysha Gene Therapies?

Institutions typically measure themselves against a benchmark when reporting to their own investors, so they often become more enthusiastic about a stock once it's included in a major index. We would expect most companies to have some institutions on the register, especially if they are growing.

Taysha Gene Therapies already has institutions on the share registry. Indeed, they own a respectable stake in the company. This can indicate that the company has a certain degree of credibility in the investment community. However, it is best to be wary of relying on the supposed validation that comes with institutional investors. They too, get it wrong sometimes. It is not uncommon to see a big share price drop if two large institutional investors try to sell out of a stock at the same time. So it is worth checking

the past earnings trajectory of Taysha Gene Therapies, (below). Of course, keep in mind that there are other factors to consider, too.



NasdaqGS:TSHA Earnings and Revenue Growth October 26th 2022

Hedge funds don't have many shares in Taysha Gene Therapies. With a 23% stake, CEO R. Session is the largest shareholder. For context, the second largest shareholder holds about 13% of the shares outstanding, followed by an ownership of 8.0% by the third-largest shareholder.

To make our study more interesting, we found that the top 5 shareholders control more than half of the company which implies that this group has considerable sway over the company's decision-making.

While it makes sense to study institutional ownership data for a company, it also makes sense to study analyst sentiments to know which way the wind is blowing. There are a reasonable number of analysts covering the stock, so it might be useful to find out their aggregate view on the future.

Insider Ownership Of Taysha Gene Therapies

While the precise definition of an insider can be subjective, almost everyone considers board members to be insiders. Management ultimately answers to the board. However,

it is not uncommon for managers to be executive board members, especially if they are a founder or the CEO.

Insider ownership is positive when it signals leadership are thinking like the true owners of the company. However, high insider ownership can also give immense power to a small group within the company. This can be negative in some circumstances.

It seems insiders own a significant proportion of Taysha Gene Therapies, Inc.. It has a market capitalization of just US\$122m, and insiders have US\$45m worth of shares in their own names. We would say this shows alignment with shareholders, but it is worth noting that the company is still quite small; some insiders may have founded the business. You can [click here to see if those insiders have been buying or selling](#).

Health Care Sector Update for 10/25/2022: HSTM, REVB, TSHA, MEDP

October 25, 2022 — 03:52 pm EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were moderately higher compared with most other sectors this afternoon, with the NYSE Health Care Index and the SPDR Health Care Select Sector ETF (XLV) both adding about 0.7% late in Tuesday trading.

The Nasdaq Biotechnology index also was climbing 2.0% this afternoon.

In company news, HealthStream ([HSTM](#)) rose 4.5% after the health care staffing firm reported Q3 net income of \$0.12 per share, more than doubling its \$0.05 per share year-ago profit and beating the five-analyst consensus call by \$0.07 per share.

Medpace Holdings ([MEDP](#)) gained over 35% after the drug and medical device development company late Monday beat analyst projections with its Q3 results and it also raised its guidance for FY22 net income and revenue above Wall Street expectations. It earned \$2.05 per share during the three months ended Sept. 30 on \$383.7 million in revenue compared with the Capital IQ consensus expecting \$1.48 per share and \$357.2 million, respectively.

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Revelation Biosciences ([REVB](#)) was trading 70% higher, easing from an early 105% advance, after Tuesday reporting positive results from a preclinical study showing its REVTx-300 drug candidate significantly lowered renal cortical fibrosis in patients with acute kidney injury and chronic kidney disease. Phase 1 testing is expected to begin during the first half of 2023.

Taysha Gene Therapies ([TSHA](#)) rallied Tuesday, climbing over 91% in afternoon trading after overnight saying Japanese drug maker Astellas Pharma will invest \$50 million to acquire a 15% stake in Taysha in addition to an exclusive option to license its TSHA-102 investigational treatment for Rett syndrome and its TSHA-120 gene therapy candidate for giant axonal neuropathy.

Health Care Sector Update for 10/25/2022: REVB,TSHA,MEDP

October 25, 2022 — 01:26 pm EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were moderately higher compared with most other sectors this afternoon, with the NYSE Health Care Index and the SPDR Health Care Select Sector ETF (XLV) both adding about 0.6%.

The Nasdaq Biotechnology index also was climbing 1.7%.

In company news, Revelation Biosciences ([REVB](#)) was trading almost 69% higher, easing from an early 105% advance, after Tuesday reporting positive results from a preclinical study showing its REVTx-300 drug candidate significantly lowered renal cortical fibrosis in patients with acute kidney injury and chronic kidney disease. Phase 1 testing is expected to begin during the first half of 2023.

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[Unsold Prefabricated Cabins](#)

Learn More

Taysha Gene Therapies ([TSHA](#)) rallied Tuesday, climbing nearly 62% in afternoon trading after overnight saying Japanese drug maker Astellas Pharma will invest \$50 million to acquire a 15% stake in Taysha in addition to an exclusive option to license its TSHA-102 investigational treatment for Rett syndrome and its TSHA-120 gene therapy candidate for giant axonal neuropathy.

Medpace Holdings ([MEDP](#)) gained over 38 after the drug and medical device development company late Monday beat analyst projections with its Q3 results and it also raised its guidance for FY22 net income and revenue above Wall Street expectations. It earned \$2.05 per share during the three months ended Sept. 30 on \$383.7 million in revenue compared with the Capital IQ consensus expecting \$1.48 per share and \$357.2 million, respectively.

Health Care Sector Update for 10/25/2022: TSHA, MEDP, CNC, XLV, IBB

October 25, 2022 — 09:11 am EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were flat to lower pre-bell Tuesday. The Health Care SPDR ([XLV](#)) was down 0.22% and the iShares Biotechnology Index ([IBB](#)) was flat.

Taysha Gene Therapies ([TSHA](#)) was gaining over 50% in value after announcing that Astellas Pharma will invest \$50 million to acquire a 15% stake in the company.

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Medpace Holdings ([MEDP](#)) was up 29% after it reported Q3 earnings of \$2.05 per diluted share, up from \$1.29 a year ago. Four analysts polled by Capital IQ expected \$1.48.

Centene ([CNC](#)) reported Q3 adjusted earnings of \$1.30 per diluted share, up from \$1.26 a year earlier. Analysts polled by Capital IQ forecast \$1.23. Centene was over 3% lower recently.

Pre-market Movers: TSHA, LMST, MEDP, WEBR, AAN...

October 25, 2022 — 08:34 am EDT

Written by RTTNews.com for [RTTNews](#) ->

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(RTTNews) - The following are some of the stocks making big moves in Tuesday's pre-market trading (as of 08.30 A.M. ET).

In the Green

Taysha Gene Therapies, Inc. (TSHA) is up over 41% at \$2.13 Limestone Bancorp, Inc. (LMST) is up over 29% at \$25.31 Medpace Holdings, Inc. (MEDP) is up over 28% at

\$203.66 Weber Inc. (WEBR) is up over 26% at \$6.36 The Aaron's Company, Inc. (AAN) is up over 12% at \$9.15

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In the Red

Crown Holdings, Inc. (CCK) is down over 10% at \$76.48 Xerox Holdings Corporation (XRX) is down over 10% at \$14.26 PepGen Inc. (PEPG) is down over 10% at \$8.49 SHF Holdings, Inc. (SHFS) is down over 10% at \$2.56 Franklin Street Properties Corp. (FSP) is down over 9% at \$2.20

TRVN To Report Data In Mid-year, TSHA Trims Workforce, FDA Decision On SPRO's NDA To Be Delayed?

April 01, 2022 — 05:31 am EDT

Written by RTTNews.com for [RTTNews](#) ->

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(RTTNews) - The following are some of the biotech companies that provided an update on their pipeline progress on Thursday.

1. Galectin Therapeutics Inc. (GALT) now expects to complete enrollment in its NAVIGATE trial by June 30, 2022.

NAVIGATE is an adaptive, two-stage, Phase 2b/3 study assessing the efficacy, safety, and tolerability of Galectin's lead drug candidate Belapectin compared with placebo for the prevention of esophageal varices in with nonalcoholic steatohepatitis (NASH) cirrhosis.

The company's cash on hand totaled \$39.6 million as of Dec.31, 2021.

GALT closed Thursday's trading at \$1.61, down 5.85%.

2. Soleno Therapeutics Inc. (SLNO), a clinical-stage biopharmaceutical company, is working towards advancing its lead candidate DCCR, a once-daily oral tablet for the treatment of Prader-Willi Syndrome, to an NDA submission.

In June 2020, the company had reported top-line results from its phase III trial of once-daily Diazoxide Choline Controlled Release (DCCR) tablets for patients with Prader-Willi Syndrome, dubbed DESTINY PWS. The study did not meet its primary endpoint of change from baseline in hyperphagia, the hallmark symptom of PWS, but showed significant improvements in prespecified subgroup with severe hyperphagia.

Last March, the FDA informed Soleno that an additional controlled clinical trial will be necessary to support an NDA submission for DCCR in Prader-Willi Syndrome. Soleno recently submitted a study proposal to the FDA and intends to start the study after an agreement with the FDA is reached.

The company's cash and cash equivalents totaled about \$21.3 million as of Dec.31, 2021.

SLNO closed Thursday's trading at \$0.22, up 2.43%.

3. Spero Therapeutics Inc. (SPRO), whose New Drug Application for Tebipenem HBr is under regulatory review in the U.S., has been notified by the FDA of certain deficiencies in the application.

The company aims to engage with the FDA to resolve the deficiencies as quickly as possible.

Tebipenem HBr, proposed for the treatment of complicated urinary tract infections, including acute pyelonephritis, caused by susceptible microorganisms, in adults, was initially assigned a [decision date of June 27, 2022](#).

SPRO closed Thursday's trading at \$8.70, up 1.05%.

4. Taysha Gene Therapies Inc. (TSHA) expects to receive a feedback from the FDA related to registration pathway for its lead drug candidate TSHA-120 for giant axonal neuropathy by mid-2022.

In January of this year, the company reported positive clinical efficacy and safety data for the high dose cohort as well as long-term durability data across all therapeutic doses of TSHA-120 in giant axonal neuropathy, a progressive neurodegenerative disease.

A phase I/II trial of TSHA-102 in Rett syndrome is underway, with preliminary data anticipated by year-end 2022.

In a bid to increase operational focus and efficiency, the company has decided to minimize activities for some ongoing clinical programs and pause all additional research and development. The company also revealed that it has reduced its workforce by 35%.

TSHA closed Thursday's trading at \$6.52, up 6.02%.

5. Trevena Inc. (TRVN) expects to report topline data from a study assessing the potential reduced effect of Olinvyk on cognitive function compared to IV morphine by mid-2022.

Olinvyk, an opioid agonist, was approved by the FDA for the management of moderate to severe acute pain in adults in September 2020.

The company is also conducting post-approval studies to further evaluate the potential impact of on respiratory, gastrointestinal and cognitive function outcomes in the postoperative setting and topline data is expected in the second half of this year.

Jiangsu Nhwa, Trevena's commercial partner in China, has sought regulatory approval for Olinvyk in China, and an application was filed in January 2022.

The company had \$66.9 million in cash and cash equivalents as of December 31, 2021.

TRVN closed Thursday's trading at \$0.54, up 24.64%.

Wall Street Analysts Believe Taysha Gene Therapies, Inc. (TSHA) Could Rally 608%: Here's is How to Trade

April 04, 2022 — 10:16 am EDT

Written by Zacks Equity Research for [Zacks ->](#)

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Shares of **Taysha Gene Therapies, Inc.** (TSHA) have gained 10.8% over the past four weeks to close the last trading session at \$6.34, but there could still be a solid upside left in the stock if short-term price targets of Wall Street analysts are any indication. Going by the price targets, the mean estimate of \$44.90 indicates a potential upside of 608.2%.

The average comprises 10 short-term price targets ranging from a low of \$33 to a high of \$60, with a standard deviation of \$8.80. While the lowest estimate indicates an increase of 420.5% from the current price level, the most optimistic estimate points to an 846.4% upside. More than the range, one should note the standard deviation here, as it helps understand the variability of the estimates. The smaller the standard deviation, the greater the agreement among analysts.

While the consensus price target is highly sought after by investors, the ability and unbiasedness of analysts in setting price targets have long been questionable. And investors making investment decisions solely based on this tool would arguably do themselves a disservice.

However, an impressive consensus price target is not the only factor that indicates a potential upside in TSHA. This view is strengthened by the agreement among analysts that the company will report better earnings than what they estimated earlier. Though a

positive trend in earnings estimate revisions doesn't give any idea as to how much the stock could surge, it has proven effective in predicting an upside.

Here's What You Should Know About Analysts' Price Targets

According to researchers at several universities across the globe, a price target is one of many pieces of information about a stock that misleads investors far more often than it guides. In fact, empirical research shows that price targets set by several analysts, irrespective of the extent of agreement, rarely indicate where the price of a stock could actually be heading.

While Wall Street analysts have deep knowledge of a company's fundamentals and the sensitivity of its business to economic and industry issues, many of them tend to set overly optimistic price targets. Are you wondering why?

They usually do that to drum up interest in shares of companies that their firms either have existing business relationships with or are looking to be associated with. In other words, business incentives of firms covering a stock often result in inflated price targets set by analysts.

However, a tight clustering of price targets, which is represented by a low standard deviation, indicates that analysts have a high degree of agreement about the direction and magnitude of a stock's price movement. While that doesn't necessarily mean the stock will hit the average price target, it could be a good starting point for further research aimed at identifying the potential fundamental driving forces.

That said, while investors should not entirely ignore price targets, making an investment decision solely based on them could lead to disappointing ROI. So, price targets should always be treated with a high degree of skepticism.

Here's Why There Could be Plenty of Upside Left in TSHA

There has been increasing optimism among analysts lately about the company's earnings prospects, as indicated by strong agreement among them in revising EPS estimates higher. And that could be a legitimate reason to expect an upside in the stock. After all, empirical research shows a strong correlation between trends in earnings estimate revisions and near-term stock price movements.

Over the last 30 days, the Zacks Consensus Estimate for the current year has increased 1.8%, as one estimate has moved higher compared to no negative revision.

Moreover, TSHA currently has a Zacks Rank #2 (Buy), which means it is in the top 20% of more than the 4,000 stocks that we rank based on four factors related to earnings estimates. Given an impressive [externally-audited track record](#), this is a more conclusive indication of the stock's potential upside in the near term. You can see [the complete list of today's Zacks Rank #1 \(Strong Buy\) stocks here >>>](#)

Therefore, while the consensus price target may not be a reliable indicator of how much TSHA could gain, the direction of price movement it implies does appear to be a good guide.

Just Released: Zacks Top 10 Stocks for 2022

In addition to the investment ideas discussed above, would you like to know about our 10 top buy-and-hold tickers for the entirety of 2022?

Last year's 2021 *Zacks Top 10 Stocks* portfolio returned gains as high as +147.7%. Now a brand-new portfolio has been handpicked from over 4,000 companies covered by the Zacks Rank. Don't miss your chance to get in on these long-term buys

Astellas Pharma To Support Development Of Taysha's AAV-based Gene Therapy Programs

October 24, 2022 — 09:14 pm EDT

Written by RTTNews.com for [RTTNews ->](#)

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(RTTNews) - Astellas Pharma Inc. (ALPMY, ALPMY) will invest in Taysha Gene Therapies Inc. (TSHA) to support the advancement of Taysha's adeno-associated virus or AAV gene therapy development programs for the treatment of Rett syndrome and giant axonal neuropathy or GAN, Taysha Gene said in a statement.

TSHA closed Monday regular trading at \$1.51 up \$0.05 or 3.42%. In the after-hours trade, the stock further gained \$0.91 or 60.26%.

As per the terms of agreement, Astellas will invest a total of \$50 million to acquire 15% of the outstanding common stock of Taysha and to receive an exclusive option to license two of Taysha's clinical stage programs: TSHA-102 for Rett syndrome and TSHA-120 for GAN.

In addition, Taysha has granted Astellas certain rights related to any potential change of control of Taysha.

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In connection with its equity investment, Astellas will receive one Board observer seat on Taysha's Board of Directors.

Taysha is engaged in the development of intrathecally-delivered AAV gene therapies for monogenic central nervous system diseases. As a part of the platform approach, Taysha has a promising pipeline, including TSHA-102, which is the first-and-only gene therapy in clinical development for Rett syndrome, and TSHA-120, which is in Phase 1/2 development for the treatment of GAN and awaiting regulatory feedback.

Pre-Market Earnings Report for May 16, 2022 : TSEM, WIX, SOHU, CMRX, GRCL, LUNA, CGEN, IMPL, TCRT, TSHA, BCLI, PASG

May 13, 2022 — 04:00 pm EDT

The following companies are expected to report earnings prior to market open on 05/16/2022. Visit our Earnings Calendar for a full list of expected earnings releases.

Tower Semiconductor Ltd. (TSEM) is reporting for the quarter ending March 31, 2022. The electric company company's consensus earnings per share forecast from the 1 analyst that follows the stock is \$0.47. This value represents a 74.07% increase compared to the same quarter last year. In the past year

TSEM has met analyst expectations twice and beat the expectations the other two quarters. Zacks Investment Research reports that the 2022 Price to Earnings ratio for TSEM is 23.69 vs. an industry ratio of 8.00, implying that they will have a higher earnings growth than their competitors in the same industry.

Wix.com Ltd. (WIX)is reporting for the quarter ending March 31, 2022. The information technology services company's consensus earnings per share forecast from the 6 analysts that follow the stock is \$-1.75. This value represents a 28.68% decrease compared to the same quarter last year. In the past year WIX has met analyst expectations once and beat the expectations the other three quarters. Zacks Investment Research reports that the 2022 Price to Earnings ratio for WIX is -11.48 vs. an industry ratio of 35.50.

Sohu.com Limited (SOHU)is reporting for the quarter ending March 31, 2022. The internet services company's consensus earnings per share forecast from the 1 analyst that follows the stock is \$-0.31. This value represents a 138.75% decrease compared to the same quarter last year. In the past year SOHU has beat the expectations every quarter. The highest one was in the 4th calendar quarter where they beat the consensus by 114.75%. Zacks Investment Research reports that the 2022 Price to Earnings ratio for SOHU is -8.48 vs. an industry ratio of 2.10.

Chimerix, Inc. (CMRX)is reporting for the quarter ending March 31, 2022. The medical products company's consensus earnings per share forecast from the 4 analysts that follow the stock is \$-0.35. This value represents a 94.44% decrease compared to the same quarter last year. Zacks Investment Research reports that the 2022 Price to Earnings ratio for CMRX is -27.33 vs. an industry ratio of -9.20.

Gracell Biotechnologies Inc. (GRCL)is reporting for the quarter ending March 31, 2022. The biomedical (gene) company's consensus earnings per share forecast from the 2 analysts that follow the stock is \$-0.08. This value represents a 68.00% increase compared to the same quarter last year. The days to cover, as reported in the 4/29/2022 short interest update, increased 160.00% from previous report on 4/14/2022. Zacks Investment Research reports that the 2022 Price to Earnings ratio for GRCL is -8.72 vs. an industry ratio of -1.10.

Luna Innovations Incorporated (LUNA)is reporting for the quarter ending March 31, 2022. The technology services company's consensus earnings per share forecast from the 2 analysts that follow the stock is \$-0.03. This value represents a 400.00% decrease compared to the same quarter last year. Zacks Investment Research reports that the 2022 Price to Earnings ratio for LUNA is 37.00 vs. an industry ratio of 6.00, implying that they will have a higher earnings growth than their competitors in the same industry.

Compugen Ltd. (CGEN)is reporting for the quarter ending March 31, 2022. The biomedical (gene) company's consensus earnings per share forecast from the 5 analysts that follow the stock is \$-0.12. This value represents a no change for the same quarter last year. In the past year CGEN has met analyst expectations once and beat the expectations the other three quarters. Zacks Investment Research reports that the 2022 Price to Earnings ratio for CGEN is -3.62 vs. an industry ratio of -1.10.

Impel Pharmaceuticals Inc. (IMPL)is reporting for the quarter ending March 31, 2022. The drug company's consensus earnings per share forecast from the 1 analyst that follows the stock is \$-1.08. This value represents a 100.00% decrease compared to the same quarter last year. Zacks Investment Research reports that the 2022 Price to Earnings ratio for IMPL is -1.61 vs. an industry ratio of 0.80.

Alaunos Therapeutics, Inc. (TCRT)is reporting for the quarter ending March 31, 2022. The drug company's consensus earnings per share forecast from the 3 analysts that follow the stock is \$-0.09. This value represents a 10.00% increase compared to the same quarter last year. In the past year TCRT has beat the expectations every quarter. The highest one was in the 4th calendar quarter where they beat the consensus by 44.44%. The "days to cover" for this stock exceeds 13 days. Zacks Investment Research reports that the 2022 Price to Earnings ratio for TCRT is -1.82 vs. an industry ratio of 0.80.

Taysha Gene Therapies, Inc. (TSHA)is reporting for the quarter ending March 31, 2022. The medical company's consensus earnings per share forecast from the 6 analysts that follow the stock is \$-1.03. This value represents a 18.39% decrease compared to the same quarter last year. Zacks Investment Research reports that the 2022 Price to Earnings ratio for TSHA is -0.82 vs. an industry ratio of 2.80.

Brainstorm Cell Therapeutics Inc. (BCLI)is reporting for the quarter ending March 31, 2022. The biomedical (gene) company's consensus earnings per share forecast from the 1 analyst that follows the stock is \$-0.13. This value represents a 31.58% increase compared to the same quarter last year. In the past year BCLI has beat the expectations every quarter. The highest one was in the 4th calendar quarter where they beat the consensus by 15%. The "days to cover" for this stock exceeds 13 days. Zacks Investment Research reports that the 2022 Price to Earnings ratio for BCLI is -4.52 vs. an industry ratio of -1.10.

Passage Bio, Inc. (PASG)is reporting for the quarter ending March 31, 2022. The biomedical (gene) company's consensus earnings per share forecast from the 4 analysts that follow the stock is \$-0.88. This value represents a 15.79% decrease compared to the same quarter last year. Zacks Investment Research reports that the 2022 Price to Earnings ratio for PASG is -0.50 vs. an industry ratio of -1.10, implying that they will have a higher earnings growth than their competitors in the same industry.

Insider Buying: The Taysha Gene Therapies, Inc. (NASDAQ:TSHA) Independent Director Just Bought 3.4% More Shares

February 10, 2022 — 09:42 am EST

Written by Simply Wall St for [Simply Wall St ->](#)

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Those following along with **Taysha Gene Therapies, Inc.** (NASDAQ:TSHA) will no doubt be intrigued by the recent purchase of shares by Paul Manning, Independent Director of the company, who spent a stonking US\$1.4m on stock at an average price of US\$7.80. While that only increased their holding size by 3.4%, it is still a big swing by our standards.

The Last 12 Months Of Insider Transactions At Taysha Gene Therapies

Notably, that recent purchase by Paul Manning is the biggest insider purchase of Taysha Gene Therapies shares that we've seen in the last year. That means that an insider was happy to buy shares at around the current price of US\$8.02. That means they have been optimistic about the company in the past, though they may have changed their mind. If someone buys shares at well below current prices, it's a good sign on balance, but keep in mind they may no longer see value. The good news for Taysha Gene Therapies share holders is that insiders were buying at near the current price.

Taysha Gene Therapies insiders may have bought shares in the last year, but they didn't sell any. The average buy price was around US\$9.58. This is nice to see since it implies

that insiders might see value around current prices. The chart below shows insider transactions (by companies and individuals) over the last year. By clicking on the graph below, you can see the precise details of each insider transaction!



NasdaqGS:TSHA Insider Trading Volume February 10th 2022

Taysha Gene Therapies is not the only stock insiders are buying. So take a peek at this free [list of growing companies with insider buying](#).

Insider Ownership

Many investors like to check how much of a company is owned by insiders. Usually, the higher the insider ownership, the more likely it is that insiders will be incentivised to build the company for the long term. Taysha Gene Therapies insiders own 43% of the company, currently worth about US\$132m based on the recent share price. This kind of significant ownership by insiders does generally increase the chance that the company is run in the interest of all shareholders.

So What Does This Data Suggest About Taysha Gene Therapies Insiders?

It is good to see recent purchasing. And an analysis of the transactions over the last year also gives us confidence. However, we note that the company didn't make a profit over the last twelve months, which makes us cautious. When combined with notable insider ownership, these factors suggest Taysha Gene Therapies insiders are well aligned, and quite possibly think the share price is too low. One for the watchlist, at least! So while it's helpful to know what insiders are doing in terms of buying or selling, it's also helpful to know the risks that a particular company is facing. When we did our research, we

found [**3 warning signs for Taysha Gene Therapies**](#) (1 shouldn't be ignored!) that we believe deserve your full attention.

If you would prefer to check out another company -- one with potentially superior financials -- then do not miss this free [list of interesting companies, that have HIGH return on equity and low debt.](#)

Taysha Gene Therapies Inc Shares Approach 52-Week Low - Market Mover



January 14, 2022 — 05:53 am EST

Written by Kwhen Finance Editors for [Kwhen ->](#)

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Taysha Gene Therapies Inc ([TSHA](#)) shares closed today at 0.5% above its 52 week low of \$9.46, giving the company a market cap of \$365M. The stock is currently down 18.4% year-to-date, down 65.9% over the past 12 months, and down 60.5% over the past five years. This week, the Dow Jones Industrial Average fell 0.3%, and the S&P 500 rose 0.6%.

Trading Activity

- Trading volume this week was 48.3% lower than the 20-day average.
- Beta, a measure of the stock's volatility relative to the overall market stands at 1.9.

Technical Indicators

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- The Relative Strength Index (RSI) on the stock was under 30, indicating it may be undebought.
- MACD, a trend-following momentum indicator, indicates a downward trend.
- The stock closed above its Bollinger band, indicating it may be overbought.

Market Comparative Performance

- The company's share price is the same as the S&P 500 Index , lags it on a 1-year basis, and lags it on a 5-year basis
- The company's share price is the same as the Dow Jones Industrial Average , lags it on a 1-year basis, and lags it on a 5-year basis

Per Group Comparative Performance

- The company's stock price performance year-to-date lags the peer average by 7541.5%
- The company's stock price performance over the past 12 months lags the peer average by 316.3%

Taysha Gene Therapies Inc Shares Approach 52-Week Low - Market Mover



January 06, 2022 — 11:45 pm EST

Written by Kwhen Finance Editors for [Kwhen](#) ->

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Taysha Gene Therapies Inc ([TSHA](#)) shares closed today at 0.9% above its 52 week low of \$10.82, giving the company a market cap of \$418M. The stock is currently down 6.6% year-to-date, down 57.1% over the past 12 months, and down 54.8% over the past five years. This week, the Dow Jones Industrial Average fell 0.2%, and the S&P 500 fell 1.9%.

Trading Activity

- Trading volume this week was 18.9% lower than the 20-day average.
- Beta, a measure of the stock's volatility relative to the overall market stands at 1.8.

Technical Indicators

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- The Relative Strength Index (RSI) on the stock was under 30, indicating it may be undebought.
- MACD, a trend-following momentum indicator, indicates a downward trend.
- The stock closed above its Bollinger band, indicating it may be overbought.

Market Comparative Performance

- The company's share price is the same as the S&P 500 Index , lags it on a 1-year basis, and lags it on a 5-year basis
- The company's share price is the same as the Dow Jones Industrial Average , lags it on a 1-year basis, and lags it on a 5-year basis

Per Group Comparative Performance

- The company's stock price performance year-to-date lags the peer average by 736.3%
- The company's stock price performance over the past 12 months lags the peer average by 87.4%

Taysha Gene Therapies Inc Shares Close in on 52-Week Low - Market Mover

January 05, 2022 — 05:51 am EST

Written by Kwhen Finance Editors for [Kwhen ->](#)



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Taysha Gene Therapies Inc ([TSHA](#)) shares closed today at 1.9% above its 52 week low of \$11.09, giving the company a market cap of \$434M. The stock is currently down 3.0% year-to-date, down 56.8% over the past 12 months, and down 53.0% over the past five years. This week, the Dow Jones Industrial Average rose 1.1%, and the S&P 500 rose 0.1%.

Trading Activity

- Trading volume this week was 55.0% lower than the 20-day average.
- Beta, a measure of the stock's volatility relative to the overall market stands at 0.0.

Technical Indicators

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- The Relative Strength Index (RSI) on the stock was under 30, indicating it may be undebought.
- MACD, a trend-following momentum indicator, indicates a downward trend.
- The stock closed above its Bollinger band, indicating it may be overbought.

Market Comparative Performance

- The company's share price is the same as the S&P 500 Index , lags it on a 1-year basis, and lags it on a 5-year basis
- The company's share price is the same as the Dow Jones Industrial Average , lags it on a 1-year basis, and lags it on a 5-year basis

Per Group Comparative Performance

- The company's stock price performance year-to-date lags the peer average by -200.8%
- The company's stock price performance over the past 12 months lags the peer average by 90.2%

Morgan Stanley: These 2 'Strong Buy' Stocks Could Double From Here

October 29, 2021 — 09:47 am EDT

Written by Michael Marcus for TipRanks ->

Two months ago, Morgan Stanley had posed a question: 'Will the September market swoon take longer than average to recover?' According to the firm's chief US equity strategist Mike Wilson, we can "Fast forward to today, and the answer to that question is a definitive no. Instead, our data show retail investors remain steadfast in their commitment to buying equities..."

Wilson sees retail investors giving a large boost to the market's current upward impetus, and paradoxically, he believes that the prospect of difficult times is motivating them. In Wilson's view, retail investors are moving into stocks as a defensive measure, recognizing that in the current economic environment of rising inflation and low interest rates, high-performance stocks provide a degree of protection for investment portfolios.

With this in mind, we wanted to take a closer look at two stocks that just received Morgan Stanley's stamp of approval, with the firm projecting upside potential of more than 100% for each. Using TipRanks' database, we found out that the rest of the Street is also on board as both have earned a "Strong Buy" consensus rating.

Taysha Gene Therapies (TSHA)

We'll start with a Texas-based biopharmaceutical company, Taysha Gene Therapies. This company is focused on developing new treatments for monogenic central nervous system (CNS) diseases. The company has an active pipeline, featuring 26 adeno-associated virus therapies. These viruses are native to humans and other primates, and are used to deliver therapeutic agents – modified genes – directly to affected cells in the patient's body. Three of Taysha's pipeline candidates are in clinical trials, while the remainder are in pre-clinical phases of development.

Of the drug candidates in clinical trials, the leader is TSHA-120. Earlier this year, the company released visual acuity data from the Phase 1/2 trials of TSHA-120 for GAN (giant axonal neuropathy, a genetic CNS disorder that manifests in early childhood). The company is expecting to receive regulatory guidance prior to additional testing before the end of this year.

Also entering the Phase 1/2 stage of clinical trials is TSHA-101, which the company announced in September of this year had received orphan drug designation from the European Commission. TSHA-101 is a treatment for infantile GM2 gangliosidosis, another CNS disease of early childhood – but one that leads to an early death, by the age of 4.

In another recent update, the company's Angelman Syndrome (AS) program was the subject of a recent published data. The company publicized positive proof-of-concept preclinical data supporting its approach to treatment of AS, a CNS disorder that can cause severe physical and mental disabilities starting in early childhood. The company is targeting UBE3A gene replacement therapy as a treatment for this disorder. Taysha is expected to start IND-enabling studies early next year, prior to human clinical trials.

Taysha's active pipeline drew Morgan Stanley's' Matthew Harrison attention. The analyst believes "Taysha's clinically validated gene therapy approach and a rapidly advancing, robust pipeline with multiple catalysts ahead, sets the stage for upside."

How much upside? Harrison rates TSHA an Overweight (i.e. Buy), and his \$39 price target implies a robust 140% one-year upside potential for the shares. (To watch Harrison's track record, click [here](#))

"We are Overweight Taysha and believe its broad AAV9 gene therapy platform, supported by management expertise in the space, has the potential to benefit patients with genetic disorders characterized by high unmet need... Taysha's pipeline includes four late-stage assets (in GAN, GM2, CLN1, and Rett syndrome) each having a risk-adjusted peak sales potential of \$1B+," Harrison opined.

The Morgan Stanley view is no outlier on this highly speculative biotech. The stock has 8 reviews on record and all are positive, for a unanimous Strong Buy consensus rating. The shares are priced at \$16.25 and their \$44.14 average price target suggests room for ~172% upside growth. (See TSHA stock analysis on TipRanks)

AlloVir (ALVR)

The second Morgan Stanley pick we'll look at is another biotech. AlloVir is focused on the treatment of viral disease, through the development of off-the-shelf, allogenic, virus-specific T-cell (VST) therapies. VSTs offer the potential to treat deadly viral diseases in patients with compromised immune systems. The company's research pipeline features five drug candidates, in various stages of development from preclinical to Phase 3 pivotal trials.

The lead candidate, posoleucel, or ALVR105, is a multi-virus specific T-cell therapy aiming at 5 separate viral diseases: BK virus (BKV), cytomegalovirus (CMV), adenovirus (AdV), Epstein-Barr virus (EBV), and human herpesvirus 6 (HHV-6). These are all potentially life-threatening disease agents, and are particularly dangerous for transplant patients. Posoleucel is designed to combat the viral agents until the patient's immune system recovers enough to take over.

Posoleucel currently has no fewer than 6 clinical trials ongoing, including a Phase 3 registrational study in virus-associated hemorrhagic cystitis and three Phase 2 proof-of-concept studies. Impressively, in the Phase 2 CHARMs study, featuring hematopoietic stem-cell transplantation patients with treatment-refractory infection, posoleucel resulted in a 93% clinical response, with activity against all target viruses, after six weeks of treatment.

"We believe the VST platform has broad potential, and is de-risked by the CHARMs data. We have added AlloVir as a Top Pick based on a favorable risk/reward ahead of Phase II multi-virus prevention data in 4Q21," said Morgan Stanley's' Matthew Harrison

Harrison rates ALVR stock an Overweight (i.e. Buy), along with a \$48 price target, showing his confidence in a 101% upside for the next 12 months.

"We are Overweight AlloVir as we believe there is significant opportunity for Viralym-M and other pipeline VSTs to meaningfully improve upon the standard of care in a number of viral indications," Harrison summed up.

All in all, other analysts echo Harrison's sentiment. 3 Buys and no Holds or Sells add up to a Strong Buy consensus rating. Given the \$44 average price target, the upside potential comes in at 84%. (See ALVR stock analysis on TipRanks)

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Unsold Prefabricated Cabins Are Being Sold On Clearance (See Prices)

Unsold Prefabricated Cabins

To find good ideas for stocks trading at attractive valuations, visit TipRanks' Best Stocks to Buy, a newly launched tool that unites all of TipRanks' equity insights.

Disclaimer: The opinions expressed in this article are solely those of the featured analysts. The content is intended to be used for informational purposes only. It is very important to do your own analysis before making any investment. Health Care Sector Update for 10/06/2021: TAK, FDMT, TSHA, XLV, IBB

October 06, 2021 — 09:11 am EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were trading lower premarket Wednesday. The Health Care SPDR ([XLV](#)) was slipping by 0.65% and the iShares NASDAQ Biotechnology Index ([IBB](#)) was recently down more than 0.9%.

Takeda Pharmaceutical ([TAK](#)) was slipping nearly 8% after saying it has suspended its mid-stage trials of its oral orexin agonist TAK-994 following a safety signal. The company said it will assess the benefit /risk profile of TAK-994 and determine the next steps while the program is suspended.

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Unsold Prefabricated Cabins Are Being Sold On Clearance (See Prices)Unsold

Prefabricated Cabins

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4D Molecular Therapeutics ([FDMT](#)) was gaining more than 3% in value after saying the US Food and Drug Administration has cleared the investigational new drug application for 4D-150 to treat wet age-related macular degeneration, paving the way for its phase 1/2 clinical trial that will be conducted before the end of the year.

Taysha Gene Therapies ([TSHA](#)) was rallying past 8% amid a filing with the US Securities and Exchange Commission for the sale of various securities totaling up to \$350 million.

Health Care Sector Update for 09/29/2021: FOLD, TSHA, LLY, XLV, IBB

September 29, 2021 — 09:15 am EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were mixed premarket Wednesday. The Health Care SPDR ([XLV](#)) was up 0.46% and the iShares NASDAQ Biotechnology Index ([IBB](#)) was recently inactive.

Amicus Therapeutics ([FOLD](#)) gained more than 12% after saying its gene therapy business will be acquired by Arya Sciences Acquisition Corp IV (ARYD), a blank-check company, sponsored by Perceptive Advisors.

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Unsold Prefabricated Cabins Are Selling Almost For Nothing! (Check It Out)Unsold

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Taysha Gene Therapies ([TSHA](#)) climbed more than 10% after saying TSHA-101, its gene replacement therapy candidate to treat a rare enzyme deficiency-related disease gangliosidosis, has received orphan drug designation from the European Commission.

Eli Lilly ([LLY](#)) was up more than 1% after saying the US Food and Drug Administration has approved the use of Erbitux in combination with Braftovi in adults with a certain type of metastatic colorectal cancer.

Busy Months Ahead For Taysha Gene

September 23, 2021 — 06:24 am EDT

Written by RTTNews.com for [RTTNews](#) ->

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(RTTNews) - Taysha Gene Therapies Inc. (TSHA), a pivotal-stage gene therapy company, has a couple of clinical, regulatory and preclinical milestones lined up for the remainder of this year.

The company has 26 AAV-based gene therapies under development for the treatment of monogenic diseases of the central nervous system, including neurodegenerative diseases, neurodevelopmental disorders, and genetic epilepsies. Monogenic disorders are caused by variation in a single gene.

The most advanced program in the pipeline is TSHA-120, an *intrathecally dosed AAV9 gene therapy, currently being evaluated in a phase I/II clinical trial for the treatment of giant axonal neuropathy (GAN). (*Intrathecal delivery refers to injection of the drug into the cerebrospinal fluid within the intrathecal space of the spinal column).

Giant axonal neuropathy is a rare and severe neurodegenerative disease. Currently, there are no approved treatments for GAN, which often results in death for patients in their late teens or early twenties. The clinical trial of TSHA-120 is being conducted by the National Institutes of Health (NIH) in close collaboration with a patient advocacy group focused on finding treatments and cures for GAN.

Human proof-of-concept data for TSHA-120 has demonstrated clear arrest of disease progression and long-term durability at therapeutic dose levels in patients with giant axonal neuropathy, according to the company.

The company expects to report clinical data from a high dose cohort of TSHA-120 in the GAN trial in the second half of this year and provide a regulatory update by year-end.

Another clinical program is TSHA-101, which is under an investigator-sponsored phase I/II trial for the treatment of infantile GM2 gangliosidosis. This trial is being conducted by Queen's University in Ontario, Canada.

Preliminary safety and biomarker data in the phase I/II trial for TSHA-101 are expected in the second half of this year. The company also expects to initiate a phase I/II clinical trial of TSHA-101 in the U.S. in the second half of 2021.

Next in the pipeline is TSHA-118 to potentially treat CLN1, a rapidly progressing rare lysosomal storage disease with no approved treatments.

The company expects to initiate a phase I/II clinical trial of TSHA-118 in the second half of this year. TSHA-118 has been granted orphan drug designation, rare pediatric disease designation and fast track designation from the FDA and orphan medicinal product designation from the EMA for the treatment of CLN1 disease.

In addition to the above, Taysha has a number of programs in preclinical stage, i.e., TSHA-102 in Rett syndrome, TSHA-104 in SURF1-associated Leigh syndrome, TSHA-105 in SLC13A5 deficiency, TSHA-111-LAFORIN and TSHA-111-MALIN in two forms of Lafora disease, TSHA-112 in APBD, TSHA-119 in GM2 AB variant and TSHA-103 in SLC6A1 haploinsufficiency disorder.

Cash position:

As of June 30, 2021, Taysha had \$197.4 million in cash and cash equivalents.

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The company made its debut on the Nasdaq Global Select Market on September 24, 2020, offering its shares at a price of \$20 each. Taysha Gene will mark one year as a public company tomorrow (Sep.24, 2021).

The stock has thus far hit a low of \$15.50 and a high of \$33.35. TSHA closed Wednesday's trading at \$18.98, up 2.54%.

Health Care Sector Update for 08/25/2021: SAVA, BBIO, TSHA, XLV, IBB

August 25, 2021 — 09:13 am EDT

Written by MT Newswires for [MTNewswires ->](#)

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Health care stocks were mixed pre-bell Wednesday as the Health Care SPDR ([XLV](#)) was 0.07% lower and the iShares NASDAQ Biotechnology Index ([IBB](#)) was recently unchanged.

Cassava Sciences ([SAVA](#)) said the US Food and Drug Administration has reviewed and agreed to the key designs of its proposed late-stage study protocols on simufilam as a potential treatment for Alzheimer's disease. Cassava Sciences was recently slumping by more than 28%.

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BridgeBio Pharma ([BBIO](#)) was gaining over 6% in value after the company and LianBio said the first patient has been dosed in a phase 2a trial of infigratinib intended to treat patients with locally advanced or metastatic gastric cancer and other solid tumors.

Taysha Gene Therapies ([TSHA](#)) was rallying past 19% after saying its therapy TSHA-105 has received the European Commission's orphan drug designation for treating epilepsy caused by mutations in the SLC13A5 gene.

Multiple insiders bought Taysha Gene Therapies, Inc. (NASDAQ:TSHA) stock earlier this year, a positive sign for shareholders

August 10, 2021 — 08:20 am EDT

Written by Simply Wall St for [Simply Wall St ->](#)

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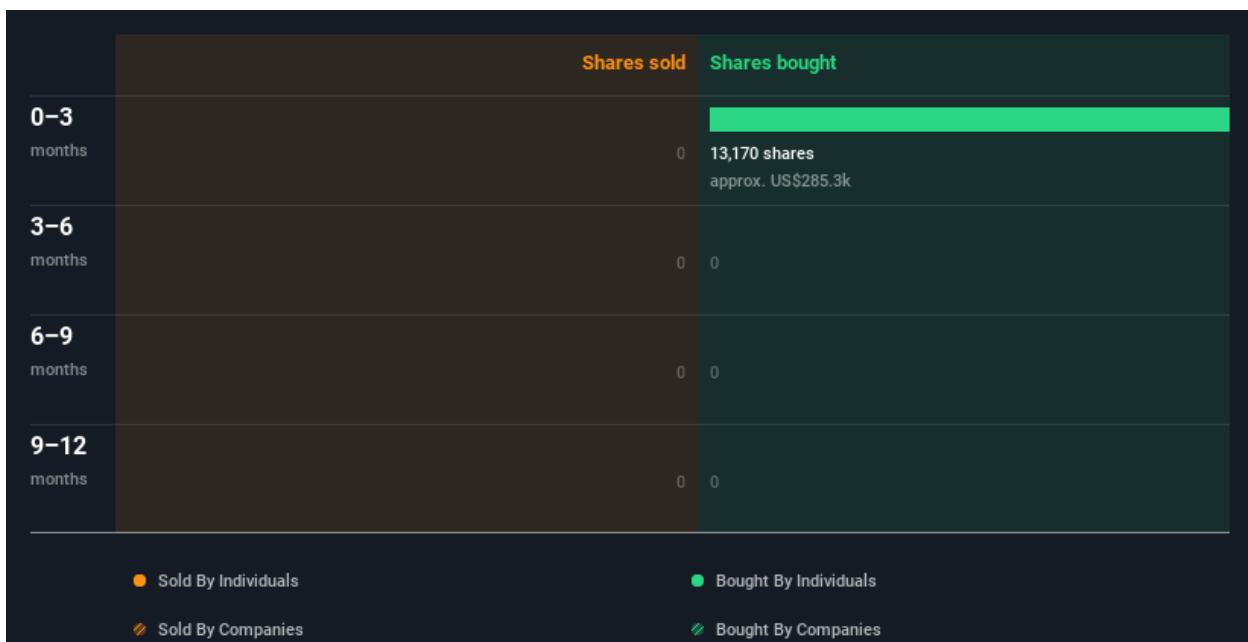
It is usually uneventful when a single insider buys stock. However, When quite a few insiders buy shares, as it happened in **Taysha Gene Therapies, Inc.'s** (NASDAQ:TSHA) case, it's fantastic news for shareholders.

While insider transactions are not the most important thing when it comes to long-term investing, we do think it is perfectly logical to keep tabs on what insiders are doing.

The Last 12 Months Of Insider Transactions At Taysha Gene Therapies

Over the last year, we can see that the biggest insider purchase was by Independent Director Paul Manning for US\$252k worth of shares, at about US\$20.98 per share. So it's clear an insider wanted to buy, even at a higher price than the current share price (being US\$17.89). While their view may have changed since the purchase was made, this does at least suggest they have had confidence in the company's future. In our view, the price an insider pays for shares is very important. As a general rule, we feel more positive about a stock if insiders have bought shares at above current prices, because that suggests they viewed the stock as good value, even at a higher price.

In the last twelve months Taysha Gene Therapies insiders were buying shares, but not selling. The chart below shows insider transactions (by companies and individuals) over the last year. By clicking on the graph below, you can see the precise details of each insider transaction!



NasdaqGS:TSHA Insider Trading Volume August 10th 2021

Taysha Gene Therapies is not the only stock that insiders are buying. For those who like to find **winning investments** this free [list of growing companies with recent insider purchasing, could be just the ticket.](#)

Insiders at Taysha Gene Therapies Have Bought Stock Recently

It's good to see that Taysha Gene Therapies insiders have made notable investments in the company's shares. In total, insiders bought US\$278k worth of shares in that time, and we didn't record any sales whatsoever. This could be interpreted as suggesting a positive outlook.

Insider Ownership

For a common shareholder, it is worth checking how many shares are held by company insiders. We usually like to see fairly high levels of insider ownership. Taysha Gene Therapies insiders own about US\$284m worth of shares (which is 42% of the company). I like to see this level of insider ownership, because it increases the chances that management are thinking about the best interests of shareholders.

So What Does This Data Suggest About Taysha Gene Therapies Insiders?

The recent insider purchases are heartening. And the longer term insider transactions also give us confidence. However, we note that the company didn't make a profit over the last twelve months, which makes us cautious. When combined with notable insider ownership, these factors suggest Taysha Gene Therapies insiders are well aligned, and quite possibly think the share price is too low. Looks promising! So these insider transactions can help us build a thesis about the stock, but it's also worthwhile knowing the risks facing this company. When we did our research, we found [**3 warning signs for Taysha Gene Therapies**](#) (1 doesn't sit too well with us!) that we believe deserve your full attention.

Of course **Taysha Gene Therapies may not be the best stock to buy**. So you may wish to see this free [collection of high quality companies](#).

What Is The Ownership Structure Like For Taysha Gene Therapies, Inc. (NASDAQ:TSHA)?

May 12, 2021 — 06:50 am EDT

Written by Simply Wall St for Simply Wall St ->

The big shareholder groups in Taysha Gene Therapies, Inc. (NASDAQ:TSHA) have power over the company. Insiders often own a large chunk of younger, smaller, companies while huge companies tend to have institutions as shareholders. I generally like to see some degree of insider ownership, even if only a little. As Nassim Nicholas Taleb said, 'Don't tell me what you think, tell me what you have in your portfolio.'

With a market capitalization of US\$794m, Taysha Gene Therapies is a small cap stock, so it might not be well known by many institutional investors. In the chart below, we can see that institutions own shares in the company. Let's delve deeper into each type of owner, to discover more about Taysha Gene Therapies.

ownership-breakdown

NasdaqGS:TSHA Ownership Breakdown May 12th 2021

What Does The Institutional Ownership Tell Us About Taysha Gene Therapies?

Institutional investors commonly compare their own returns to the returns of a commonly followed index. So they generally do consider buying larger companies that are included in the relevant benchmark index.

We can see that Taysha Gene Therapies does have institutional investors; and they hold a good portion of the company's stock. This implies the analysts working for those institutions have looked at the stock and they like it. But just like anyone else, they could be wrong. When multiple institutions own a stock, there's always a risk that they are in a 'crowded trade'. When such a trade goes wrong, multiple parties may compete to sell stock fast. This risk is higher in a company without a history of growth. You can see Taysha Gene Therapies' historic earnings and revenue below, but keep in mind there's always more to the story.

earnings-and-revenue-growth

NasdaqGS:TSHA Earnings and Revenue Growth May 12th 2021

Hedge funds don't have many shares in Taysha Gene Therapies. Looking at our data, we can see that the largest shareholder is the CEO R. Session with 25% of shares outstanding. With 15% and 14% of the shares outstanding respectively, FMR LLC and Paul Manning are the second and third largest shareholders. Interestingly, the third-largest shareholder, Paul Manning is also a Member of the Board of Directors, again, indicating strong insider ownership amongst the company's top shareholders.

A more detailed study of the shareholder registry showed us that 3 of the top shareholders have a considerable amount of ownership in the company, via their 54% stake.

Researching institutional ownership is a good way to gauge and filter a stock's expected performance. The same can be achieved by studying analyst sentiments. There are a reasonable number of analysts covering the stock, so it might be useful to find out their aggregate view on the future.

Insider Ownership Of Taysha Gene Therapies

While the precise definition of an insider can be subjective, almost everyone considers board members to be insiders. The company management answer to the board and the latter should represent the interests of shareholders. Notably, sometimes top-level managers are on the board themselves.

Most consider insider ownership a positive because it can indicate the board is well aligned with other shareholders. However, on some occasions too much power is concentrated within this group.

It seems insiders own a significant proportion of Taysha Gene Therapies, Inc.. It has a market capitalization of just US\$794m, and insiders have US\$308m worth of shares in their own names. It is great to see insiders so invested in the business. It might be worth checking if those insiders have been buying recently.

General Public Ownership

With a 12% ownership, the general public have some degree of sway over Taysha Gene Therapies. While this group can't necessarily call the shots, it can certainly have a real influence on how the company is run.

Next Steps:

While it is well worth considering the different groups that own a company, there are other factors that are even more important. Consider risks, for instance. Every company has them, and we've spotted 2 warning signs for Taysha Gene Therapies you should know about.

If you would prefer discover what analysts are predicting in terms of future growth, do not miss this free report on analyst forecasts.

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[Unsold Prefabricated Cabins Are Being Sold On Clearance \(See Prices\)](#)

[Unsold Prefabricated Cabins](#)

NB: Figures in this article are calculated using data from the last twelve months, which refer to the 12-month period ending on the last date of the month the financial statement is dated. This may not be consistent with full year annual report figures.

This article by Simply Wall St is general in nature. It does not constitute a recommendation to buy or sell any stock, and does not take account of your objectives, or your financial situation. We aim to bring you long-term focused analysis driven by fundamental data. Note that our analysis may not factor in the latest price-sensitive company announcements or qualitative material. Simply Wall St has no position in any stocks mentioned.

BUZZ-U.S. STOCKS ON THE MOVE- Twitter, Caterpillar, Wells Fargo, Restaurant Brands



Credit: REUTERS/LUCAS JACKSON
October 14, 2020 — 01:39 pm EDT

Written by [Reuters ->](#)

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Eikon search string for individual stock moves: STXBZ

The Day Ahead newsletter: <http://tmsnrt.rs/2ggOmBi>

The Morning News Call newsletter: <http://tmsnrt.rs/2fwPLTh>

Wall Street's main indexes fell on Wednesday as Treasury Secretary Steven Mnuchin said it was difficult that a deal on more fiscal stimulus would be reached before the presidential election in November. .N

At 13:09 ET, the Dow Jones Industrial Average .DJI was down 0.36% at 28,575.79, the S&P 500 .SPX was down 0.58% at 3,491.72, and the Nasdaq Composite .IXIC was down 0.90% at 11,756.664. The top three S&P 500 .PG.INX percentage gainers: ** Concho Resources Inc , up 12.3% ** Baker Hughes Co , up 5.4% ** National Oilwell Varco Inc , up 4.6% The top three S&P 500 .PL.INX percentage losers: ** Wells Fargo & Co , down 5.4% ** Kroger Co , down 4.6% ** Illumina Inc , down 4.5% The top three NYSE .PG.N percentage gainers: ** Nio Inc , up 21% ** Concho Resources Inc , up 12.3% ** Oil States International Inc , up 11.7% The top three NYSE .PL.N percentage losers: ** Navistar International Corp , down 18.5% ** AMC Entertainment Holdings Inc , down 16.8% ** Enzo Biochem Inc , down 15.5% The top three Nasdaq .PG.O percentage gainers: ** Medalist Diversified REIT Inc , up 204.7% ** Interpace Biosciences Inc , up 41.9% ** Replimune Group Inc , up 29.7% The top three Nasdaq .PL.O percentage losers: ** Cycleron Therapeutics Inc , down 50.1% ** Codiak BioSciences Inc , down 16.9% ** Sorrento Therapeutics Inc , down 13.3% ** Concho Resources CXO.N: up 12.3% BUZZ- Rises on report ConocoPhillips in takeover talks ** AMC Entertainment Holdings Inc AMC.N: down 16.8% BUZZ- AMC flags possible equity raise, shares slump ** Allscripts Healthcare Solutions Inc MDRX.O: up 29.5% BUZZ- Surges after agreeing to sell CarePort Health unit for \$1.35 bln ** Bank of America BAC.N: down 4.2% BUZZ- Falls as Q3 profit drops on COVID-19 hit

** Goldman Sachs Group GS.N: up 1.4% BUZZ- Rises as Q3 profit nearly doubles due to trading surge ** Novan NOVN.O: up 33.6% BUZZ- Rises after treatment shows promise in treating COVID-19 ** Bentley Systems Inc BSY.O: up 10.0% BUZZ- Berenberg sees scope to turn on "growth engine" ** Interpace Biosciences IDXG.O: up 41.9% BUZZ- Interpace Biosciences rises as probe finds no evidence of illegal acts ** Safe-T Group SFET.O: up 3.6% BUZZ- Jumps on upbeat Q3 revenue outlook ** Humanigen HGEN.O: up 17.9% BUZZ- HC Wainwright begins coverage with 'buy', shares rise ** Nio NIO.N: up 21.0% BUZZ- Jumps after JP Morgan turns bullish, triples PT to

Street-high ** Nautilus NLS.N: up 0.9% BUZZ- Gains after Truist Securities lifts PT on strong demand outlook ** SS&C Technologies SSNC.O: up 1.4% BUZZ- D.A. Davidson raises PT on hopes of upbeat Q3 results ** Energy Focus EFOI.O: up 2.7% BUZZ- Surges on launching advanced disinfection products

** Taiwan Liposome TLC.O: up 3.7% BUZZ- Up on starting early-stage study of COVID-19 treatment ** Lazydays Holdings Inc LAZY.O: down 13.3%

BUZZ- Slides on stock offering plans ** Moderna MRNA.O: down 2.0%

BUZZ-Moderna gets EU nod to submit COVID-19 vaccine marketing application, shares rise ** Vericel Corp VCEL.O: up 0.6%

BUZZ- Preliminary Q3 revenue up 5%, shares rise ** cbdMD YCBD.A: up 0.4%

BUZZ- Up as FY sales likely to double on lockdown demand

** Wells Fargo WFC.N: down 5.4%

BUZZ- Falls as Q3 profit misses Street estimate ** Harmony Biosciences Holdings HRMY.O: up 7.6%

BUZZ- Up as FDA approves expanded use of sleep disorder drug

** Boeing BA.N: up 1.3%

BUZZ- Rises as brokerage sees grounding of 737 MAX lifting in Q4 ** Pilgrim's Pride PPC.O: up 6.2%

BUZZ- Up after settling price-fixing case with DOJ ** American Eagle Outfitters AEO.N: up 2.1% ** Abercrombie & Fitch ANF.N: up 1.4%

BUZZ-American Eagle, Abercrombie & Fitch gain as B. Riley lifts PTs ** Taysha Gene Therapies TSHA.O: up 1.2%

BUZZ- Rises on rare pediatric, orphan drug tags for Rett syndrome therapy ** Caterpillar CAT.N: up 0.7%

BUZZ- Hits over 2-1/2 year high after reaffirming qtrly dividend ** Dave & Buster's PLAY.O: up 6.6%

BUZZ- Rises on signs of same-store sales recovery ** Waddell & Reed WDR.N: up 7.1%

BUZZ- Gains on reports of takeover speculation ** Westlake Chemical WLK.N: up 3.3%

BUZZ- Climbs as brokerages see gains from higher PVC prices ** Alaska Air ALK.N: up 3.2%

BUZZ- Gains as revenue decline slows in September ** Codiak BioSciences Inc CDAK.O: down 16.9%

BUZZ- Falls in Nasdaq debut ** Twitter Inc TWTR.N: down 2.0%

BUZZ- Dips as lax security faulted for July cyberattack ** Navistar NAV.N: down 18.5%

BUZZ- Drops as Traton's buyout offer to expire on Oct. 16 ** Restaurant Brands QSR.N: up 1.0%

BUZZ-Restaurant Brands rises on strong EBITDA forecast, sales recovery ** VOXX International VOXX.O: up 24.4%

BUZZ- Jumps after co swings to Q2 profit

The 11 major S&P 500 sectors:

Communication Services

.SPLRCL

down 1.18%

Consumer Discretionary

.SPLRCD

down 1.39%

Consumer Staples

.SPLRCS

down 0.51%

Energy

.SPNY

up 1.90%

Financial

.SPSY

down 0.29%

Health

.SPXHC

down 0.66%

Industrial

.SPLRCI

up 0.90%

Information Technology

.SPLRCT

down 0.82%

Materials

.SPLRCM

up 0.62%

Real Estate

.SPLRCR

down 0.54%

Utilities

.SPLRCU

down 0.26%

BUZZ-U.S. STOCKS ON THE MOVE- Abercrombie & Fitch, Boeing, Wells Fargo, Goldman Sachs



Credit: REUTERS/LUCAS JACKSON
October 14, 2020 — 12:02 pm EDT

Written by [Reuters ->](#)

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Eikon search string for individual stock moves: STXBZ

The Day Ahead newsletter: <http://tmsnrt.rs/2ggOmBi>

The Morning News Call newsletter: <http://tmsnrt.rs/2fwPLTh>

Wall Street's main indexes inched higher in choppy trading on Wednesday as investors digested a mixed bag of quarterly earnings reports from major U.S. banks. .N

At 11:43 a.m. ET, the Dow Jones Industrial Average .DJI was down 0.12% at 28,644.05. The S&P 500 .SPX was down 0.09% at 3,508.77 and the Nasdaq Composite .IXIC was down 0.11% at 11,850.79. The top three S&P 500 .PG.INX percentage gainers: ** Concho Resources Inc , up 13.6% ** Apache Corp , up 8.6% ** Diamondback Energy Inc , up 6.3% The top three S&P 500 .PL.INX percentage losers: ** Wells Fargo & Co , down 4.8% ** Bank Of America , down 3.8% ** T Mobile US Inc , down 3.3% The top three NYSE .PG.N percentage gainers: ** Nio Inc , up 21.9% ** Nabors Inds Ltd , up 14.9% ** Concho Resources Inc , up 13.6% The top three NYSE .PL.N percentage losers: ** AMC Entertainment Holdings Inc , down 14.5% ** Enzo Biochem Inc , down 13.8% ** Navios Maritime Holdings Inc , down 9.6% The top three Nasdaq .PG.O percentage gainers: ** Medalist Diversified REIT Inc , up 205% ** Interpace Biosciences Inc , up 43.9% ** Allscripts Healthcare Solutions Inc , up 31.9% The top three Nasdaq .PL.O percentage losers: ** Cycleron Therapeutics Inc , down 48.4% ** Codiak BioSciences Inc , down 17% ** Sorrento Therapeutics Inc , down 15% ** Theratechnologies THTX.O: down 0.4% BUZZ- Soars on U.S. patent for NASH disease treatment ** Concho Resources CXO.N: up 13.6% BUZZ- Rises on report ConocoPhillips in takeover talks ** Apple Inc AAPL.O: up 0.3% BUZZ-Street View: Customers will be keen to jump on Apple's iPhone 5G bandwagon ** AMC Entertainment Holdings Inc AMC.N: down 14.5% BUZZ-AMC flags possible equity raise, shares slump ** Allscripts Healthcare Solutions Inc MDRX.O: up 31.9% BUZZ- Surges after agreeing to sell CarePort Health unit for \$1.35 bln ** Bank of America BAC.N: down 3.8% BUZZ- Fall as Q3 profit drops on COVID-19 hit

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Consumer Discretionary

.SPLRCD

down 0.74%

Consumer Staples

.SPLRCS

down 0.31%

Energy

.SPNY

up 2.60%

Financial

.SPSY

down 0.13%

Health

.SPXHC

down 0.20%

Industrial

.SPLRCI

up 1.06%

Information Technology

.SPLRCT

down 0.13%

Materials

.SPLRCM

up 0.91%

Real Estate

.SPLRCR

down 0.93%

Utilities

.SPLRCU

up 0.01%

BUZZ-U.S. STOCKS ON THE MOVE-Universal Insurance Holdings, Square Inc, Taysha Gene Therapies Inc

Credit: REUTERS/BRENDAN MCDERMID

September 24, 2020 — 01:19 pm EDT

Written by Reuters ->

Eikon search string for individual stock moves: STXBZ

The Day Ahead newsletter: <http://tmsnrt.rs/2ggOmBi>

The Morning News Call newsletter: <http://tmsnrt.rs/2fwPLTh>

Wall Street climbed in choppy trading on Thursday, with investors returning to the perceived safety of technology-related stocks as a surprise rise in weekly jobless claims signaled a slowdown in economic growth. .N

At 13:00 ET, the Dow Jones Industrial Average .DJI was up 0.78% at 26,971.44. The S&P 500 .SPX was up 0.85% at 3,264.43 and the Nasdaq Composite .IXIC was up 1.06% at 10,745.421. The top three S&P 500 .PG.INX percentage gainers: ** Darden Restaurants Inc , up 9.6% ** Goldman Sachs Group , up 5.2% ** Albemarle Corp , up 4.5% The top three S&P 500 .PL.INX percentage losers: ** Carmax Inc , down 9.8% ** Accenture Plc , down 6.8% ** Twitter Inc , down 4.4% The top three NYSE .PG.N percentage gainers: ** Owens & Minor Inc , up 55.4% ** Methode Electronics Inc MEI.N, up 14.6% ** CorEnergy Infrastructure Trust Inc , up 10.6% The top three NYSE .PL.N percentage losers: ** Rite Aid Corp , down 15.3% ** Renren Inc RENN.N, down 12.2% ** Laird Superfood Inc , down 12% The top three Nasdaq .PG.O percentage gainers: ** Sunworks Inc , up 224.6% ** Peck Company Holdings Inc , up 143.4% ** Polar Power Inc , up 68.7% The top three Nasdaq .PL.O percentage losers: ** Golar LNG Ltd , down 27.3% ** Digital Ally Inc , down 25.5% ** Kubient Inc , down 25.2% ** Nikola Corporation NKLA.O: down 4.7%

BUZZ-Wedbush sees cloudy path to 2023; downgrades ** E.W. Scripps Co SSP.O: up 11.4%

BUZZ-Surges on deal to buy ION Media for \$2.65 bln ** Switchback Energy Acquisition Corp SBE.N: up 5.6%

BUZZ-Soars on deal to take ChargePoint public ** Sunworks Inc SUNW.O: up 224.6% ** Polar Power Inc POLA.O: up 68.7%

BUZZ-Sunworks, Polar Power surge on California's plans to ban gasoline-vehicles from 2035 ** EnPro Industries Inc NPO.N: up 6.3%

BUZZ-KeyBanc upgrades on portfolio transformation ** SPI Energy Co Ltd SPI.O: up 14.3%

BUZZ-No stopping the surge: SPI Energy extends gains on launch of EV unit ** Rockwell Automation Inc ROK.N: up 2.2%

BUZZ-Daiwa upgrades to 'outperform' on growing EV popularity ** Penn National Gaming Inc PENN.O: down 6.6%

BUZZ-Slides on planned 14 mln share offering ** Leap Therapeutics Inc LPTX.O: up 1.4%

BUZZ-Rises on FDA 'fast track' tag for cancer treatment ** Broadcom Inc AVGO.O: up 1.1%

BUZZ-Piper Sandler raises PT on growth prospects ** Co-Diagnostics Inc CODX.O: up 1.9%

BUZZ-Rises on plan to launch COVID-19 test kits in October ** Darden Restaurants Inc DRI.N: up 9.6%

BUZZ-Rises as Olive Garden operator sees sales improvement ** Jefferies Financial Group Inc JEF.N: up 9.1%

BUZZ-Gains as Q3 revenue jumps ** Kingsoft Cloud Holdings Ltd KC.O: down 7.6%

BUZZ-Dips after pricing equity offering ** Johnson & Johnson JNJ.N: up 0.4%

BUZZ-J&J's COVID-19 vaccine on track to get emergency use nod in 2021 - analysts ** Alphabet Inc GOOGL.O: up 1.2%

BUZZ-Morgan Stanley hikes PT on potential upside from e-commerce ** Kubient Inc KBNT.O: down 25.2%

BUZZ-Slumps on bigger Q2 loss as COVID-19 hits business ** Owens & Minor Inc OMI.N: up 55.3%

BUZZ-Raises profit forecast on PPE kit demand, shares soar ** Accenture PLC ACN.N: down 6.8%

BUZZ-Set to open at 2-month low on forecast miss ** BlackBerry Ltd BB.N: up 5.2%

BUZZ-Gains as software demand drives Q2 results beat ** FedEx Corp FDX.N: up 1.2%

BUZZ-Stifel upgrades to 'buy', says a pandemic winner ** Jabil Inc JBL.N: up 5.3%

BUZZ-Rises on upbeat forecast for Q1 of FY21 ** Dynatronics Corp DYNT.O: down 8.0%

BUZZ-Drops on pandemic-driven quarterly loss ** Crexendo Inc CXDO.O: down 11.2%

BUZZ-Sinks further after pricing stock offering ** AMC Entertainment Holdings Inc AMC.N: down 2.4%

BUZZ-Drops on plans to raise capital via share sale ** Millendo Therapeutics Inc MLND.O: up 0.3%

BUZZ-Rises on dosing first patient in hot flashes therapy study ** Titan Pharmaceuticals Inc TTNP.O: down 13.1%

BUZZ-Tumbles on registered direct offering ** Drive Shack Inc DS.N: up 4.5%

BUZZ-Gains after co names new CFO ** CarMax Inc KMX.N: down 9.8%

BUZZ-Falls as used car inventory drops amid demand rebound ** Armada Hoffler Properties Inc AHH.N: up 3.2%

BUZZ-Gains on signaling improving rent collections ** Methode Electronics Inc MEI.N: up 14.6%

BUZZ-Raises Q2 outlook as automotive demand improves ** Medtronic PLC MDT.N: down 0.8%

BUZZ-Truist cuts PT; says co signals rise in operating expenses ** Taysha Gene Therapies Inc TSHA.O: up 18.8%

BUZZ-Gain 11% in Nasdaq debut ** Universal Insurance Holdings Inc UVE.N: down 1.7%

BUZZ-Down as Piper Sandler cuts PT ** Gilead Sciences Inc GILD.O: down 1.4%

BUZZ-Lots of partnership interest for Immunomedics prior to deal, says Jefferies ** Square Inc SQ.N: up 2.5%

BUZZ-Gains as Oppenheimer upgrades stock to 'outperform' ** Factset Research Systems Inc FDS.N: up 4.1%

BUZZ-FactSet rises on quarterly results beat, upbeat forecast

The 11 major S&P 500 sectors:

Communication Services

.SPLRCL

up 0.78%

Consumer Discretionary

.SPLRCD

up 0.93%

Consumer Staples

.SPLRCS

up 0.90%

Energy

.SPNY

up 0.21%

Financial

.SPSY

up 0.84%

Health

.SPXHC

down 0.17%

Industrial

.SPLRCI

up 0.56%

Information Technology

.SPLRCT

up 1.43%

Materials

.SPLRCM

up 0.85%

Real Estate

.SPLRCR

up 1.35%

Utilities

.SPLRCU

**up 1.41% Preclinical CNS biotech Taysha
Gene Therapies prices upsized IPO at \$20
high end**

September 24, 2020 — 07:36 am EDT

Written by Renaissance Capital for Renaissance Capital ->



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Taysha Gene Therapies, a preclinical biotech developing gene therapies for very rare CNS disorders, raised \$157 million by offering 7.9 million shares at \$20, the high end of the range of \$18 to \$20. The company offered 1.3 million more shares than anticipated. At pricing, the company raised 26% more in proceeds than expected.

Taysha Gene Therapies plans to list on the Nasdaq under the symbol TSHA. Goldman Sachs, Morgan Stanley and Jefferies acted as lead managers on the deal.

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The article [Preclinical CNS biotech Taysha Gene Therapies prices upsized IPO at \\$20 high end](#) originally appeared on IPO investment manager Renaissance Capital's web site [renaissancecapital.com](#).

Investment Disclosure: The information and opinions expressed herein were prepared by Renaissance Capital's research analysts and do not constitute an offer to buy or sell any security. Renaissance Capital's [Renaissance IPO ETF \(symbol: IPO\)](#), [Renaissance International ETF \(symbol: IPOS\)](#), or separately managed institutional accounts may have investments in securities of companies mentioned.

Taysha Gene Therapies to Participate in Upcoming Chardan Genetic Medicines and Cell Therapy Manufacturing Summit

Apr. 18, 2022 8:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Chief Executive Officer, Chief Technical Officer, Chief Medical Officer and Head of Research and Development and Senior Vice President of Manufacturing to participate in a panel discussion at the Chardan Genetic Medicines and Cell Therapy Manufacturing Summit on April 25, 2022 at 12:00 pm ET

Chardan Genetic Medicines and Cell Therapy Manufacturing Summit Fireside Chat on April 25, 2022 at 1:00 pm ET

DALLAS, April 18, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced its participation in the upcoming panel and fireside chat at the Chardan Genetic Medicines and Cell Therapy Manufacturing Summit.

Conference Details:

Event: Chardan Genetic Medicines and Cell Therapy Manufacturing Summit

Topic: Evolving standards of AAV GT manufacturing – learnings from experience in registration-targeted trials

Date: Monday, April 25, 2022

Time: 12:00 pm ET

Format: Panel Discussion

Participants: RA Session II, President, Founder and CEO

Dr. Suyash Prasad, Chief Medical Officer and Head of R&D

Dr. Frederick Porter, Chief Technical Officer

Greg Gara, SVP, Manufacturing

Conference Details:

Event: Chardan Genetic Medicines and Cell Therapy Manufacturing Summit

Date: Monday, April 25, 2022

Time: 1:00 pm ET

Format: Fireside Chat

Participants: RA Session II, President, Founder and CEO

Dr. Suyash Prasad, Chief Medical Officer and Head of R&D

Kamran Alam, Chief Financial Officer

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Oral Presentations at the Upcoming 2022 IRSF Rett Syndrome Scientific Symposium and the ASCEND National Summit

Apr. 25, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, April 25, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced oral presentations at the upcoming International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Symposium taking place in Nashville, Tennessee April 26 – 27, 2022, and the ASCEND Rett Syndrome National Summit taking place in Nashville, Tennessee April 27 – 30, 2022.

IRSF Scientific Symposium Oral Presentation Details

- *Safety and Biodistribution Assessment in Non-human Primates (NHPs) of a miniMECP2 AAV9 Vector for Gene-replacement Therapy of Rett Syndrome*
Presenter – Dr. Suyash Prasad, Taysha Gene Therapies
Date/Time – Tuesday, April 26th at 3:45 PM Central Time
- *Rett Syndrome in Adulthood: The Caregiver Perspective*
Presenter – Kristin Phillips, Taysha Gene Therapies
Date/Time – Wednesday, April 27th at 2:00 PM Central Time

ASCEND National Summit Oral Presentation Details

- *Putting Patients at the Center*
Presenters – Dr. Suyash Prasad, Taysha Gene Therapies and Emily McGinnis,

Taysha Gene Therapies

Date/Time – Thursday, April 28th at 12:45 PM Central Time

Additional details can be found at the [IRSF Rett Syndrome Scientific Meeting website](#) and the [ASCEND Rett Syndrome National Summit website](#).

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Receives Orphan Drug Designation from the European Commission for TSHA-120 for the Treatment of Giant Axon Neuropathy (GAN)

May 03, 2022 7:00 AM ET | Taysha Gene Therapies, Inc. (TSHA)

Clinical efficacy data for TSHA-120 provide quantitative evidence of long-term durability across all therapeutic dose cohorts with a 10-point improvement in mean change in MFM32 by Year 3 compared to estimated natural history decline of 24 points

Biopsy data in five of six patient samples analyzed to date confirmed active regeneration of nerve fibers following treatment with TSHA-120

53 patient-years of clinical data support favorable safety and tolerability profile of TSHA-120

Estimated addressable patient population of 5,000 worldwide represents a multi-billion dollar commercial opportunity

No approved treatments for the underlying cause of the disease

DALLAS, May 03, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it has been granted orphan drug designation from the European Commission for TSHA-120, an intrathecally dosed AAV9 gene therapy currently in ongoing clinical evaluation for the treatment of giant axonal neuropathy (GAN).

"GAN is a progressive and devastating neurodegenerative disease that has an estimated addressable patient population of 5,000 worldwide. The disease impacts a broad range of patients, with an early onset form of the disease affecting young infants, while a late onset can affect patients into adulthood," said Suyash Prasad, MBBS, M.SC., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. "In January we announced promising data for TSHA-120, our most advanced program, demonstrating long-term durability for all three therapeutic dose cohorts and clinically significant improvements in MFM32 over time compared to decline in patients observed in natural history studies. The long-term safety and tolerability of TSHA-120 was supported by 53-patient years of data, and importantly, biopsy data confirmed active nerve fiber regeneration following treatment with TSHA-120. We are pleased to receive orphan drug designation by the European commission which can help facilitate rapid clinical advancement and subsequent access to patients as we further approach regulatory approval."

GAN is a rare inherited genetic disorder that is a progressive neurodegenerative disease that affects both the central and peripheral nervous systems. The disease is caused by loss-of-function mutations in the gene coding for *gigaxonin*, which results in dysregulation of intermediate filament turnover, an important structural component of the cell. Children with GAN present before the age of five with symptoms including unsteady gait, frequent falls, motor weakness. Currently, there are no approved treatments for GAN, which results in death for patients in their late teens or early twenties.

TSHA-120, an intrathecally dosed AAV9 gene replacement therapy delivering the gene *gigaxonin* for the treatment of GAN is currently being evaluated in an ongoing clinical trial conducted by the National Institute of Neurological Disorders and Stroke (NINDS) division of the National Institutes of Health (NIH) under the leadership of principal investigator, Carsten Bönneman, M.D. Taysha has partnered with GeneDx to support inclusion of the genetic marker for GAN in the GeneDx hereditary neuropathy panel at no cost to individuals at risk for or suspected of having GAN, and with the Hereditary Neuropathy Foundation and Charcot-Marie-Tooth Association Centers of Excellence to increase GAN disease awareness and access to testing. TSHA-120 has previously received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA).

The European Commission grants orphan drug designation for medicines being developed for the diagnosis, prevention or treatment of treat life-threatening or chronically debilitating conditions that affect fewer than 5 in 10,000 people in the European Union ([EXIT-OLD](#)). Orphan designation in the European Union includes benefits such as protocol assistance, reduced regulatory fees and market exclusivity.

About Taysha Gene Therapies

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Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, including TSHA-120, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, TSHA-120's eligibility for accelerated approval in the United States and Europe, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, and the potential market opportunity for these product candidates. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2021, which is available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Presentations at the Upcoming 25th Annual Meeting of the American Society of Gene & Cell Therapy

May 10, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, May 10, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced oral and poster presentations and a company-sponsored symposium at the upcoming 25th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) taking place in Washington, D.C. from May 16-19, 2022.

Oral Presentations

- *3: Vagus Nerve Delivery of AAV9 to Treat Autonomic Nervous System Dysfunction in Giant Axonal Neuropathy*
Presenter – Rachel Bailey, UT Southwestern
Date/Time – Monday, May 16th at 10:45 AM Eastern Time
Location – Room 204
- *470: Vectorized Delivery of Tau Reduction Therapy as a Treatment Approach for Tauopathies*
Presenter – Rachel Bailey, UT Southwestern
Date/Time – Tuesday, May 17th at 4:15 PM Eastern Time
Location – Rm 204

Poster Presentations

- *157: Preclinical Gene Therapy with AAV9/SLC6A1 in a Mouse Model of SLC6A1 Related Disorder*
Presenter – Weirui Guo, UT Southwestern
Date/Time – Monday, May 16th at 5:30 PM Eastern Time
Location – Poster Board M-38, Hall D
- *179: shRNA-Mediated Gene Therapy for the Treatment of Prader-Willi Syndrome*
Presenter – Violeta Zaric, UT Southwestern
Date/Time – Monday, May 16th at 5:30 PM Eastern Time
Location – Poster Board M-60, Hall D
- *640: Gene Therapy Treatment in Young SLC13A5 Deficient Mice*
Presenter – Rachel Bailey, UT Southwestern
Date/Time – Tuesday, May 17th at 5:30 PM Eastern Time
Location – Poster Board Tu-145, Hall D
- *557: shRNA-Mediated Gene Therapy for the Treatment of Angelman Syndrome*
Presenter – Hye Ri Kang, UT Southwestern
Date/Time – Tuesday, May 17th at 5:30 PM Eastern Time
Location – Poster Board Tu-62, Hall D
- *642: Safety and Biodistribution Assessment in Non-Human Primates (NHPs) of a miniMECP2 AAV9 Vector for Gene-Replacement Therapy of Rett Syndrome*
Presenter – Dr. Suyash Prasad, Taysha Gene Therapies
Date/Time – Tuesday, May 17th at 5:30 PM Eastern Time
Location – Poster board Tu-147, Hall D
- *1037: Safety Assessment of High-Dose miniMECP2 AAV9 Gene-Replacement Therapy (TSHA-102) for Rett Syndrome in Rats*
Presenter – Mary Newman, Taysha Gene Therapies
Date/Time – Wednesday, May 18th at 5:30 PM Eastern Time
Location –Poster board W-163, Hall D

Taysha-Sponsored Symposium

- *Accepting the Challenge: Innovative Approaches and Translational Strategies in Gene Therapy Development*

Presenters – Dr. Suyash Prasad, Taysha Gene Therapies, Dr. Steven Gray, UT Southwestern, and Dr. Kimberly Goodspeed, UT Southwestern
Date/Time – Tuesday, May 17th at 12:00 PM Eastern Time

Additional details can be found at the ASGCT 25th Annual Meeting [website](#).

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Release First Quarter 2022 Financial Results and Host Conference Call and Webcast on May 16

May 11, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, May 11, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it will report its financial results for the first quarter ended March 31, 2022, and host a corporate update conference call and webcast on Monday, May 16, 2022, at 8:00 AM Eastern Time.

Conference Call Details

Monday, May 16, at 8:00 AM Eastern Time / 7:00 AM Central Time

Toll Free: 877-407-0792

International: 201-689-8263

Conference ID: 13729044

Webcast: <https://ir.tayshagtx.com/news-events/events-presentations>

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

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

May 16, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Q1: 2022-05-16 Earnings Summary

Transcript

10-Q

EPS of -\$1.31 misses by \$0.21 | Revenue of \$0.00 beats by \$0.00

Initiated clinical development of TSHA-102 for Rett Syndrome under recently approved Clinical Trial Application (CTA) with preliminary Phase 1/2 data expected by year-end 2022

Received Orphan Drug Designation from the European Commission for TSHA-120 for giant axonal neuropathy (GAN) and recently completed commercially representative GMP batch; regulatory update expected in mid-2022

Existing cash and cash equivalents, along with full access to the term loan facility, is expected to fund operating expenses and capital requirements into the fourth quarter of 2023

DALLAS, May 16, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today reported financial results for the first quarter ended March 31, 2022 and provided a corporate update.

"In 2022, we are focused on advancing our key programs in Rett syndrome and GAN. We initiated clinical development of TSHA-102 in Rett syndrome and expect preliminary clinical data from the REVEAL study by year-end," said RA Session II, President, Founder and CEO of Taysha. "Recently, the European Commission granted orphan drug designation for TSHA-120 for GAN, further highlighting the unmet need for treatment options for these patients and the important potential of TSHA-120. We have completed a commercially representative GMP batch for TSHA-120 with release testing currently underway. Our existing capital resources, along with full access to the term loan facility, should fund operating expenses and capital requirements into the fourth quarter of 2023."

Recent Corporate Highlights

TSHA-120 for giant axonal neuropathy (GAN): an intrathecally dosed AAV9 gene therapy currently being evaluated in a clinical trial for the treatment of GAN, a rare inherited genetic disorder that affects both the central and peripheral nervous systems and is caused by loss-of-function mutations in the gene coding for *gigaxonin*. TSHA-120 is designed to deliver a functional copy of the GAN gene to the CNS and PNS. TSHA-120 has already received orphan drug and rare pediatric disease designations from the U.S. Food and Drug Administration (FDA) and orphan drug designation from the European Commission.

- Received orphan drug designation from the European Commission for GAN in April 2022
- Reported positive clinical efficacy and safety data for high dose cohort and long-term durability data across all therapeutic dose cohorts for TSHA-120 in GAN
 - Efficacy data for high dose cohort demonstrated clinically meaningful and statistically significant improvement in MFM32 by Year 1 compared to natural history (n=3)

- Long-term durability data across all therapeutic dose cohorts demonstrated a 10-point improvement in mean change in MFM32 by Year 3 compared to estimated natural history decline of 24 points (n=5)
 - Biopsy data in five of six patient samples analyzed to date confirmed active regeneration of nerve fibers following treatment with TSHA-120 (n=6)
 - 53 patient-years of clinical data support the safety and tolerability profile of TSHA-120
-
- Commercially representative GMP batch completed and release testing underway
 - Regulatory feedback for TSHA-120 in GAN expected mid-2022

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

- CTA approved by Health Canada in March 2022
- Published preclinical data for TSHA-102 in Rett syndrome presented at the International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting and the ASCEND Rett Syndrome National Summit in April 2022
- Initiation of clinical development with the REVEAL study, an open-label, dose escalation, randomized, multicenter Phase 1/2 clinical trial in adult female patients with Rett syndrome
 - Sainte-Justine Mother and Child University Hospital Center in Montreal, Quebec, Canada selected as initial clinical site under the direction of Dr. Elsa Rossignol, principal investigator
 - Key assessments to include Rett-specific and global assessments, quality of life, biomarkers and neurophysiology and imaging

- Preliminary clinical data for TSHA-102 in Rett syndrome expected by year-end 2022

AAV9 Gene Replacement for CLN7 Batten disease: an investigational AAV9 intrathecally dosed gene replacement therapy designed to deliver a full-length copy of the *CLN7* gene to potentially treat CLN7 disease, a rapidly progressing rare lysosomal storage disease with no approved treatments. The clinical development of the CLN7 program is being funded by UT Southwestern (UTSW), Children's Health and Children's Medical Center Foundation.

- Reported positive preliminary clinical safety data for first-generation construct in CLN7 Batten disease from UTSW-sponsored clinical trial
 - Data from three patients dosed presented at the 18th Annual WORLD Symposium
 - Fourth patient with CLN7 disease dosed at 1.0×10^{15} total vg

Anticipated Milestones

- Regulatory update for TSHA-120 in GAN by mid-2022
- Preliminary clinical data from the REVEAL study for TSHA-102 in Rett syndrome by year-end 2022
- Initiation of clinical development for TSHA-105 in SLC13A5 deficiency
- Continued clinical development of the first-generation construct for CLN7 disease in 2022
- Continued clinical development for TSHA-118 in CLN1 disease

First Quarter 2022 Financial Highlights

Research and Development (R&D) Expenses: Research and development expenses were \$37.8 million for the three months ended March 31, 2022, compared to \$23.9 million for the three months ended March 31, 2021. The increase of approximately \$13.9 million was primarily attributable to an increase of \$9.3 million in employee compensation, which included \$2.2 million of severance and one-time termination costs in connection with the strategic reprioritization of programs completed in March 2022 and \$1.0 million of non-cash stock-based compensation. Additionally, in the three months ended March 31, 2022, we incurred an increase of \$2.9 million of expenses in research and development manufacturing and other raw material purchases. We also incurred an increase of \$1.7 million of third-party research and development consulting fees, primarily related to GLP toxicology studies and clinical study activities.

General and Administrative (G&A) Expenses: General and administrative expenses were \$11.5 million for the three months ended March 31, 2022, compared to \$8.2 million for the three months ended March 31, 2021. The increase of approximately \$3.3 million was primarily attributable to \$2.9 million of incremental compensation expense, which included \$0.4 million of severance and one-time termination costs and \$0.7 million of non-cash stock-based compensation. We also incurred an increase of \$0.4 million in professional fees related to insurance, investor relations/communications, accounting, and market research.

Net loss: Net loss for the three months ended March 31, 2022 was \$50.1 million, or \$1.31 per share, as compared to a net loss of \$32.0 million, or \$0.87 per share, for the three months ended March 31, 2021.

Cash and cash equivalents: As of March 31, 2022, we had \$96.6 million in cash and cash equivalents. This excludes approximately \$12 million in gross proceeds generated from the sale of common stock, par value \$0.00001 per share, under our existing at-the-market facility in April 2022.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 8:00 am ET / 7:00 am CT to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13729044. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, including our preclinical product candidates, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, the potential market opportunity for these product candidates, our corporate growth plans, the forecast of our cash runway and the implementation and potential impacts of our strategic pipeline prioritization initiatives. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2021 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2022, both of which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

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

For the Three Months
Ended March 31,

	2022	2021
Operating expenses:		
Research and development	\$ 37,799	\$ 23,854
General and administrative	11,469	8,236
Total operating expenses	49,268	32,090
Loss from operations	(49,268)	(32,090)
Other income (expense):		
Interest income	14	66
Interest expense	(849)	-
Other expense	(8)	-
Total other expense, net	(843)	66
Net loss	\$ (50,111)	\$ (32,024)
Net loss per common share, basic and diluted	\$ (1.31)	\$ (0.87)
Weighted average common shares outstanding, basic and diluted	38,174,717	36,992,377

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

	March 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 96,630	\$ 149,103
Prepaid expenses and other current assets	10,261	10,499
Total current assets	<u>106,891</u>	<u>159,602</u>
Restricted cash	2,637	2,637
Deferred lease asset	655	667
Property, plant and equipment, net	55,120	50,610
Other non-current assets	673	440
Total assets	<u>\$ 165,976</u>	<u>\$ 213,956</u>
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	\$ 21,997	\$ 21,763
Accrued expenses and other current liabilities	<u>26,620</u>	<u>29,983</u>
Total current liabilities	<u>48,617</u>	<u>51,746</u>
Build-to-suit lease liability	25,752	25,900
Term Loan, net	37,386	37,192
Other non-current liabilities	3,496	3,735
Total liabilities	<u>115,251</u>	<u>118,573</u>

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, 2022 and December 31, 2021

Common stock, \$0.00001 par value per share; 200,000,000 shares authorized and 38,473,945 issued and outstanding as of March 31, 2022 and December 31, 2021

Additional paid-in capital		336,485	331,032
Accumulated deficit		(285,760)	(235,649)
Total stockholders' equity		50,725	95,383
Total liabilities and stockholders' equity	\$	165,976	\$ 213,956

Company Contact:

Kimberly Lee, D.O.

Chief Corporate Affairs Officer

Taysha Gene Therapies

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Media Contact:

Carolyn Hawley

Canale Communications

carolyn.hawley@canalecomm.com



Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Participate in Upcoming June Investor Conferences and Convention

Jun. 02, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Jefferies Healthcare Conference on June 8, 2022 at 9:30 am ET

Goldman Sachs 43rd Annual Global Healthcare Conference on June 14, 2022 at 11:20 am PT

BIO International Convention on June 15, 2022 at 11:00 am PT

JMP Securities Life Sciences Conference on June 16, 2022 at 1:00 pm ET

DALLAS, June 02, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced its participation in a fireside chat at the Jefferies Healthcare Conference, Goldman Sachs 43rd Annual Global Healthcare Conference, and JMP Securities Life Sciences Conference. Additionally, RA Session II, CEO, President and Founder, will participate in a panel discussion at the BIO International Convention.

A webcast of the fireside chats will be available in the “Events & Media” section of the Taysha corporate website at <https://ir.tayshagtx.com/news-events/events-presentations>. Archived versions of the webcast will be available on the website for 90 days.

Conference Details:

Event: Jefferies Healthcare Conference

Date: Wednesday, June 8

Time: 9:30 am ET

Format: Fireside chat

Event: Goldman Sachs 43rd Annual Healthcare Conference

Date: Tuesday, June 14

Time: 11:20 am PT

Format: Fireside chat

Event: BIO International Convention

Date: Wednesday, June 15

Time: 11:00 am PT

Format: Panel discussion

Topic: "A New Era in Gene Therapy"

Event: JMP Securities Life Sciences Conference

Date: Thursday, June 16

Time: 1:00 pm ET

Format: Fireside chat

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Annual Stockholder Meeting

Jun. 13, 2022 8:39 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

GlobeNewswire

DALLAS, June 13, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that its annual stockholder meeting will be held on Friday, June 17, 2022 at 10 am ET via webcast.

The webcast for this meeting will be available in the "[Events & Presentations](#)" section of the Taysha corporate website. A copy of Taysha's 2021 annual report is available [here](#) and in the "Financial Information" section of the Taysha Corporate Website.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Company Contact:

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Chief Corporate Affairs Officer

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Media Contact:

Carolyn Hawley

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Participate in Upcoming William Blair Biotech Focus Conference 2022

Jul. 06, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

GlobeNewswire

DALLAS, July 06, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced its participation in a panel discussion on Wednesday, July 13th at the upcoming William Blair Biotech Focus Conference 2022.

A webcast of the event will be available in the “Events & Media” section of the Taysha corporate website at <https://ir.tayshagtx.com/news-events/events-presentations>. Archived versions of the webcasts will be available on the website for 60 days.

Conference Details:

Event: William Blair Biotech Focus Conference 2022

Topic: AAV Gene Therapy: Delivery and Beyond

Date: Wednesday, July 13, 2022

Time: 10:00 am ET

Format: Panel Discussion

Participants: Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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

Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Participate in Upcoming August Investor Conferences

Aug. 01, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

BTIG Biotechnology Conference on August 8, 2022 at 12:00 pm ET

2022 Wedbush PacGrow Healthcare Virtual Conference on August 10, 2022 at 9:45 am ET

DALLAS, Aug. 01, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced its participation in panel discussions at the BTIG Biotechnology Conference on August 8th and the 2022 Wedbush PacGrow Healthcare Virtual Conference on August 10th.

A webcast of the panels will be available in the “Events & Media” section of the Taysha corporate website at <https://ir.tayshagtx.com/news-events/events-presentations>. Archived versions of the webcast will be available on the website for 90 days.

Conference Details:

Event:	<i>BTIG Biotechnology Conference</i>
Topic:	AAV Gene Therapies for CNS Disorders: Challenges and Opportunities
Date:	Monday August 8
Time:	12:00 pm ET
Format:	Panel discussion
Participant:	Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFFPM, Chief Medical Officer and Head of Research and Development

Event: *2022 Wedbush PacGrow Healthcare Virtual Conference*

Topic: UltraOrphan: When You're One in a Million

Date: Wednesday, August 10

Time: 9:45 am ET

Format: Panel discussion

Participant: RA Session II, President, Founder and Chief Executive Officer

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Release Second Quarter 2022 Financial Results and Host Conference Call and Webcast on August 11

Aug. 09, 2022 5:22 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, Aug. 09, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it will report its financial results for the second quarter ended June 30, 2022, and host a corporate update conference call and webcast on Thursday, August 11, 2022, at 8:00 AM Eastern Time.

Conference Call Details

Thursday, August 11, at 8:00 AM Eastern Time / 7:00 AM Central Time

Toll Free: 877-407-0792

International: 201-689-8263

Conference ID: 13730848

Webcast: <https://ir.tayshagtx.com/news-events/events-presentations>

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

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

Aug. 11, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Q2: 2022-08-11 Earnings Summary

Transcript

10-Q

EPS of -\$0.84 beats by \$0.22 | Revenue of \$0.00 beats by \$0.00

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

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

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

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

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

DALLAS, Aug. 11, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today reported financial results for the second quarter ended June 30, 2022, and provided a corporate update.

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

Recent Corporate Highlights

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

- TSHA-120-treated patients with GAN demonstrated durable neurophysiological improvements in the sensory nerve action potential (SNAP), a definitive clinical endpoint, compared to rapid and irreversible decline in sensory function early in life in untreated patients based on natural history
 - Natural history data suggest that sensory function is unrecoverable once SNAP reaches zero
 - All patients who were 9 years and older in the natural history study demonstrated zero SNAP response

- Treatment with TSHA-120 resulted in stabilization and improvement of sensory nerve function with durability of improvement in SNAP
- Pathology from nerve biopsies in all evaluable samples confirmed the presence of regenerative nerve fibers in 100% of TSHA-120-treated patients (n=11)
- TSHA-120 commercial grade and clinical trial material considered comparable across all key quality attributes as assessed by an extensive panel of release assays and next-generation sequencing
- Positive regulatory feedback from MHRA supports regulatory strategy
 - MHRA found functional clinical data, eye pathology and visual acuity, and nerve biopsy data compelling. Agency agreed with commercial manufacturing and release testing strategy, including potency assays and recommended dosing a few patients with commercial grade material, which will be released in September 2022. MHRA was supportive of Taysha's proposal to perform validation work on MFM32 as a key clinical endpoint

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

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

Anticipated 2022 Milestones

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

Second Quarter 2022 Financial Highlights

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

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

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

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

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast with slides today at 8:00 am ET / 7:00 am CT to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13730848. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

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Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, including our preclinical product candidates, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, the potential market opportunity for these product candidates, our corporate growth plans, the forecast of our cash runway and the implementation and potential impacts of our strategic pipeline prioritization initiatives. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2021, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, both of which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

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

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

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

	June 30, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 66,239	\$ 149,103
Prepaid expenses and other current assets	<u>10,596</u>	<u>10,499</u>
Total current assets	<u>76,835</u>	<u>159,602</u>
Restricted cash	2,637	2,637
Deferred lease asset	643	667
Property, plant and equipment, net	61,011	50,610
Other non-current assets	1,206	440
Total assets	\$ 142,332	\$ 213,956
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	\$ 23,967	\$ 21,763
Accrued expenses and other current liabilities	<u>18,986</u>	<u>29,983</u>
Total current liabilities	<u>42,953</u>	<u>51,746</u>
Build-to-suit lease liability	25,609	25,900
Term Loan, net	37,580	37,192
Other non-current liabilities	3,480	3,735
Total liabilities	109,622	118,573

Stockholders' equity

Preferred stock, \$0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of June 30, 2022 and December 31, 2021

- -

Common stock, \$0.00001 par value per share; 200,000,000 shares authorized and 41,020,086 and 38,473,945 issued and outstanding as of June 30, 2022 and December 31, 2021, respectively

1 -

Additional paid-in capital	352,342	331,032
Accumulated deficit	(319,633)	(235,649)
Total stockholders' equity	32,710	95,383
Total liabilities and stockholders' equity	\$ 142,332	\$ 213,956

Company Contact:

Kimberly Lee, D.O.

Chief Corporate Affairs Officer

Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Presentations at the Upcoming 29th Annual Meeting of the European Society of Gene & Cell Therapy (ESGCT)

Oct. 06, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, Oct. 06, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced two poster presentations at the upcoming 29th Annual Meeting of the European Society of Gene & Cell Therapy (ESGCT) in cooperation with the British Society of Gene & Cell Therapy (BSGCT) taking place in Edinburgh, Scotland from October 11-14, 2022.

Poster Presentation Details

- P238: *Assessment of Safety and Biodistribution of a miniMECP2 AAV9 Vector for Gene-replacement Therapy of Rett Syndrome in Non-human Primates (NHPs)*

Presenter – Dirk Schmitt, Senior Director of Medical Affairs, Taysha Gene Therapies

Date and Time – Thursday, October 13th at 17:30 GMT + 1/12:30 PM Eastern Time
Location – Cromdale Hall

- P206: *Assessment of Safety of miniMECP2 AAV9 vector (TSHA-102) for Gene-replacement Therapy of Rett Syndrome in Rats*

Presenter – Dirk Schmitt, Senior Director of Medical Affairs, Taysha Gene Therapies

Date and Time – Thursday, October 13th at 17:30 GMT + 1/12:30 PM Eastern Time
Location – Cromdale Hall

Additional details can be found at the ESGCT 29th Annual Meeting [website](#).

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Astellas and Taysha Gene Therapies Announce Strategic Investment to Support Development of Taysha's AAV-based Gene Therapy Programs

Oct. 24, 2022 7:30 PM ET | **Astellas Pharma Inc. (ALPMY), TSHA**

- *Taysha Gene Therapies ([TSHA](#)) is an emerging leader in the development of AAV gene therapies; new collaboration aimed at enhancing development of two of Taysha's novel product candidates for rare monogenic central nervous system diseases with serious unmet medical needs -*
- *Astellas to invest a total of \$50 million to acquire 15% of the company and to receive an exclusive option to obtain an exclusive license for TSHA-102 for Rett syndrome and TSHA-120 for giant axonal neuropathy (GAN) -*
- *Astellas to receive certain rights related to any potential change of control of Taysha -*
- *Astellas to receive one Board observer seat on the Taysha Board of Directors -*

TOKYO and DALLAS, Oct. 24, 2022 (GLOBE NEWSWIRE) -- Astellas Pharma Inc. ([ALPMF](#)) (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") and Taysha Gene Therapies, Inc. today announced a strategic investment to support the advancement of Taysha's adeno-associated virus (AAV) gene therapy development programs for the treatment of Rett syndrome and GAN. The future options to potentially apply Astellas' global R&D, manufacturing and commercialization capabilities in gene therapy to Taysha's innovative AAV gene therapy development programs for genetic diseases of the central nervous system (CNS) create the opportunity for the two companies to enhance the development of novel treatment options for patients with Rett syndrome and GAN, who have serious unmet medical needs.

Under the terms of the agreement, Astellas will invest a total of \$50 million to acquire 15% of the outstanding common stock of Taysha and to receive an exclusive option to license two of Taysha's clinical stage programs: TSHA-102 for Rett syndrome and TSHA-120 for GAN. In addition, Taysha has granted Astellas certain rights related to any potential change of control of Taysha. Definitive agreements would be executed upon Astellas' exercise of any such option, and any change of control transaction would require approval by Taysha's stockholders.

Taysha is engaged in the development of intrathecally-delivered AAV gene therapies for monogenic CNS diseases. As a part of this platform approach, Taysha has a promising pipeline, including TSHA-102, which is the first-and-only gene therapy in clinical development for Rett syndrome, and TSHA-120, which is in Phase 1/2 development for the treatment of GAN and awaiting regulatory feedback.

Astellas is continuing to build its capability to bring novel gene therapies to patients, following the acquisition of Audentes (now Astellas Gene Therapies, California) in January 2020 and the construction of a state-of-the-art commercial GMP manufacturing facility in North Carolina, which was opened in June of this year.

"Gene therapy is the corner stone of Astellas' Primary Focus, Genetic Regulation^{*1}; our goal is to bring new transformative treatment options to patients living with serious genetic diseases and limited treatment options," said Naoki Okamura, Chief Strategy Officer, at Astellas. "Taysha is an industry leader in CNS gene therapies and this partnership fits strategically with our long-term vision of expanding Astellas' gene therapy capabilities, allowing the company to impact the lives of a broader range of patients with urgent unmet medical needs."

"We are excited to enter this strategic investment with Astellas, a premier biopharmaceutical company with global R&D, manufacturing and commercial capabilities," said RA Session II, Taysha's Chief Executive Officer. "We believe this investment not only further validates the potential of our technology platform, but also reinforces the therapeutic and market opportunity of our two lead clinical assets."

To further strategically align Astellas and Taysha, in connection with its equity investment, Astellas will receive one Board observer seat on Taysha's Board of Directors, enabling Taysha to leverage Astellas' gene therapy clinical and commercial expertise as Taysha advances TSHA-120 and TSHA-102.

*1: Astellas has established a Focus Area Approach for its research and development strategy. For more information, please visit our website at <https://www.astellas.com/en/science/focus-area-approach>.

About TSHA-102

TSHA-102 is a self-complementary intrathecally delivered AAV9 gene replacement therapy under development for the treatment of Rett syndrome. TSHA-102 utilizes the novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform to regulate transgene expression genotypically on a cell-by-cell basis. The miRARE technology is designed to prevent toxicity associated with transgene overexpression and can be potentially utilized across other indications. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) and Orphan Drug Designation from the European Commission.

About Rett Syndrome

Rett syndrome is a severe genetic neurodevelopmental disorder caused by a mutation in the X-linked *MECP2* gene essential for neuronal and synaptic function in the brain. Primarily occurring in females, Rett syndrome is one of the most common genetic causes of severe intellectual disability worldwide. Patients have normal early development, with symptom onset typically beginning between 6 to 18 months of age. Rett syndrome is characterized by rapid developmental regression that leads to intellectual disabilities, loss of speech, loss of purposeful use of hands, loss of mobility, seizures, cardiac impairments and breathing issues. Currently, there are no approved therapies that treat the underlying cause of this progressive disease.

About TSHA-120

TSHA-120, an intrathecally dosed AAV9 gene replacement therapy delivering the gene *gigaxonin* for the treatment of GAN, is currently being evaluated in an ongoing Phase 1/2 clinical trial. TSHA-120 has received Orphan Drug and Rare Pediatric Disease designations from FDA and Orphan Drug Designation from the European Commission.

About Giant Axonal Neuropathy (GAN)

GAN is rare inherited genetic disorder that is a progressive neurodegenerative disease that affects both the central and peripheral nervous systems. The disease is caused by loss-of-function mutations in the gene coding for *gigaxonin*, which results in dysregulation of intermediate filament turnover, an important structural component of the cell. Children with GAN present before the age of five with symptoms including unsteady gait, frequent falls, and motor weakness. Currently, there are no approved treatments for GAN, which results in death for patients in their late teens or early twenties.

About Taysha

Taysha Gene Therapies (Nasdaq: TSHA) is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements (Taysha)

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About Astellas

Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions that combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into value for patients. For more information, please visit our website at <https://www.astellas.com/en>.

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Astellas Gene Therapies is an Astellas Center of Excellence developing genetic medicines with the potential to deliver transformative value for patients. Our gene therapy drug discovery engine is built around innovative science, a validated AAV platform, and industry leading internal manufacturing capability with a particular focus on rare diseases of the eye, CNS and neuromuscular system. Astellas Gene Therapies will also be advancing additional Astellas gene therapy programs toward clinical investigation. Astellas Gene Therapies is based in San Francisco, with manufacturing and laboratory facilities in South San Francisco, Calif., and Sanford, N.C.

Astellas Cautionary Notes

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View original content to download multimedia:<https://www.prnewswire.com/news-releases/astellas-and-taysha-gene-therapies-announce-strategic-investment-to-support-development-of-tayshas-aav-based-gene-therapy-programs-301656986.html>

SOURCE Astellas Pharma Inc.

Taysha Gene Therapies to Host Conference Call to Discuss Astellas Pharma's Strategic Investment to Support the Development of Taysha's AAV-based Gene Therapy Programs

Oct. 24, 2022 7:35 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

GlobeNewswire

DALLAS, Oct. 24, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it will host a conference call and webcast with slides to discuss Astellas Pharma's strategic investment to support the development of two of Taysha's AAV-based gene therapy programs, TSHA-102 for Rett syndrome and TSHA-120 for giant axonal neuropathy (GAN), on Tuesday, October 25, 2022, at 8:00 AM Eastern Time.

Conference Call Details

Tuesday, October 25, at 8:00 AM Eastern Time / 7:00 AM Central Time

Toll Free: 877-407-0792

International: 201-689-8263

Conference ID: 13734026

Webcast: <https://ir.tayshagtx.com/news-events/events-presentations>

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Proposed Public Offering of Common Stock

Oct. 25, 2022 4:01 PM ET | [Taysha Gene Therapies, Inc. \(TSHA\)](#)

DALLAS, Oct. 25, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it intends to offer and sell, subject to market conditions, \$30 million of shares of its common stock in an underwritten public offering. All shares of common stock to be sold in the offering will be offered by Taysha. Taysha also intends to grant the underwriter a 30-day option to purchase up to an additional 15% of the shares of its common stock offered in the public offering under the same terms and conditions. The offering is subject to market conditions, and there can be no assurance as to whether or when the offering may be completed, or the actual size or terms of the offering.

Goldman Sachs & Co. LLC is acting as sole book-runner for the offering.

A shelf registration statement relating to the shares of common stock offered in the public offering described above was filed with the Securities and Exchange Commission (the "SEC") on October 5, 2021 and declared effective by the SEC on October 14, 2021. The offering will be made only by means of a written prospectus and prospectus supplement that form a part of the registration statement. A preliminary prospectus supplement and accompanying prospectus relating to the offering will be filed with the SEC and will be available on the SEC's website at www.sec.gov. Copies of the preliminary prospectus supplement and the accompanying prospectus, when available, may also be obtained by contacting Goldman Sachs & Co. LLC, Attention: Prospectus Department, 200 West Street, New York, NY 10282, or by telephone at (866) 471-2526, or by email at prospectus-ny@ny.email.gs.com.

This press release shall not constitute an offer to sell or the solicitation of an offer to buy the securities being offered, nor shall there be any sale of the securities being offered in any state or other jurisdiction in which such offer, solicitation or sale would be unlawful prior to the registration or qualification under the securities laws of any such state or other jurisdiction.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Announces Pricing of Public Offering of Common Stock

Oct. 26, 2022 7:54 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, Oct. 26, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)) ("Taysha"), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced the pricing of an underwritten public offering of 14,000,000 shares of its common stock at a price to the public of \$2.00 per share. In addition, Taysha has granted the underwriter a 30-day option to purchase up to 2,100,000 additional shares of common stock at the public offering price, less the underwriting discount. The gross proceeds from the offering to Taysha are expected to be approximately \$28.0 million, before deducting underwriting discounts and commissions and other offering expenses, excluding any exercise of the underwriter's option to purchase additional shares. The offering is expected to close on or about October 31, 2022, subject to customary closing conditions.

Goldman Sachs & Co. LLC is acting as sole book-runner for the offering.

A shelf registration statement relating to the shares of common stock offered in the public offering described above was filed with the Securities and Exchange Commission (the “SEC”) on October 5, 2021 and declared effective by the SEC on October 14, 2021. The offering is being made only by means of a written prospectus and prospectus supplement that form a part of the registration statement. A preliminary prospectus supplement and accompanying prospectus relating to the offering has been filed with the SEC and is available on the SEC’s website at www.sec.gov. A final prospectus supplement and accompanying prospectus will be filed with the SEC. When available, copies of the final prospectus supplement and the accompanying prospectus may also be obtained by contacting Goldman Sachs & Co. LLC, Attention: Prospectus Department, 200 West Street, New York, NY 10282, or by telephone at (866) 471-2526, or by email at prospectus-ny@ny.email.gs.com.

This press release shall not constitute an offer to sell or the solicitation of an offer to buy the securities being offered, nor shall there be any sale of the securities being offered in any state or other jurisdiction in which such offer, solicitation or sale would be unlawful prior to the registration or qualification under the securities laws of any such state or other jurisdiction.

About Taysha Gene Therapies, Inc.

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, Taysha aims to rapidly translate its treatments from bench to bedside. Taysha has combined its team’s proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, Taysha leverages its fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients’ lives.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of Taysha's candidates, including its preclinical product candidates, to positively impact quality of life and alter the course of disease in the patients Taysha seeks to treat, its research, development and regulatory plans for its product candidates, and Taysha's public offering, including expected gross proceeds and anticipated closing date, the uncertainties related to market conditions and the completion of the public offering on the anticipated terms or at all. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding Taysha's business are described in detail in its SEC filings, including in Taysha's Annual Report on Form 10-K for the full-year ended December 31, 2021, and Taysha's Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, both of which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that Taysha makes from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and Taysha disclaims any obligation to update these statements except as may be required by law.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Release Third Quarter 2022 Financial Results and Host Conference Call and Webcast on November 8

Nov. 02, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, Nov. 02, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it will report its financial results for the third quarter ended September 30, 2022, and host a corporate update conference call and webcast on Tuesday, November 8, 2022, at 8:00 AM Eastern Time.

Conference Call Details

Tuesday, November 8, at 8:00 AM Eastern Time / 7:00 AM Central Time

Toll Free: 855-327-6387

International: 631-891-4304

Conference ID: 10020611

Webcast: <https://ir.tayshagtx.com/news-events/events-presentations>

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

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

Nov. 08, 2022 7:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Q3: 2022-11-08 Earnings Summary

Transcript

10-Q

EPS of -\$0.64 beats by \$0.18 | Revenue of \$0.00 beats by \$0.00

\$50 million strategic investment from Astellas Pharma to support development of TSHA-120 in giant axonal neuropathy (GAN) and TSHA-102 in Rett syndrome

Proceeds from follow-on offering along with Astellas strategic investment extends cash runway into first quarter of 2024

Type B end-of-Phase 2 meeting with FDA for TSHA-120 in GAN scheduled for December 13, 2022

Preliminary clinical safety and efficacy data for TSHA-102 from the first cohort of adult patients with Rett syndrome and initiation of female pediatric study expected in first half of 2023

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

DALLAS, Nov. 08, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today reported financial results for the third quarter ended September 30, 2022 and provided a corporate update.

"The strategic investment from Astellas and the successfully completed public follow-on offering have strengthened our balance sheet and extended our cash runway into the first quarter of 2024. The partnership with Astellas underscores the therapeutic and market opportunity of our two lead clinical programs in GAN and Rett syndrome. Importantly, this strategic investment further validates our platform and our scientific approach of combining established gene therapy technology with innovative targeted payload design," said RA Session II, President, Founder and CEO of Taysha. "In 2023, we expect to provide an update on the regulatory pathway for TSHA-120 in GAN in January following our Type B end-of-Phase 2 meeting with the FDA. In addition, we intend to disclose preliminary clinical data for TSHA-102 from the first cohort of adult patients with Rett syndrome and initiate a Phase 1/2 trial for TSHA-102 in female pediatric patients with Rett syndrome in the first half of 2023."

Recent Corporate Highlights

Strategic investment from Astellas Pharma validates Taysha's scientific approach and capabilities as well as reinforces therapeutic and market opportunity of two lead programs

Astellas to invest a total of \$50 million for 15% of Taysha to support development of TSHA-120 in giant axonal neuropathy (GAN) and TSHA-102 in Rett syndrome and to receive:

- An exclusive option to obtain an exclusive license for worldwide development, manufacturing and commercial rights to:
 - TSHA-120 in GAN for a period of time after receipt of the formal Type B end-of-Phase 2 meeting minutes
 - TSHA-102 in Rett syndrome for a period of time after the company provides Astellas access to certain clinical data from the female pediatric study

- Right of first offer related to any potential change in control of Taysha for a period of time upon receipt of the Rett data package
- One non-voting Board observer seat on the Taysha Board of Directors

Pricing of public offering of common stock

- On October 26, 2022, Taysha raised \$28.0 million in gross proceeds through an underwritten public offering of 14,000,000 shares of its common stock at a price to the public of \$2.00 per share.

Anticipated Upcoming Milestones

- Regulatory update for TSHA-120 in GAN following receipt of formal meeting minutes from the Type B end-of-Phase 2 meeting with FDA expected in mid-January 2023
- Preliminary clinical safety and efficacy data for TSHA-102 from the entire first cohort of adult patients with Rett syndrome expected in H1 2023
- Initiation of Phase 1/2 trial for TSHA-102 in female pediatric patients with Rett syndrome in H1 2023

Third Quarter 2022 Financial Highlights

Research and Development (R&D) Expenses: Research and development expenses were \$16.4 million for the three months ended September 30, 2022, compared to \$39.5 million for the three months ended September 30, 2021. The \$23.1 million decrease was attributable to a decrease of \$11.7 million in research and development GMP manufacturing and other raw material purchases. Additionally, we incurred \$6.7 million less expense in third-party research and development consulting fees, primarily related to non-clinical GLP toxicology studies, and a decrease of \$4.7 million in employee compensation expenses.

General and Administrative (G&A) Expenses: General and administrative expenses were \$8.7 million for the three months ended September 30, 2022, compared to \$11.2 million for the three months ended September 30, 2021. The decrease of approximately \$2.5 million was primarily attributable to a decrease of \$1.3 million in professional fees related to pre-commercialization, recruiting and patient advocacy activities. Additionally, compensation expense decreased by \$1.2 million compared to the same period in 2021.

Net loss: Net loss for the three months ended September 30, 2022, was \$26.3 million, or \$0.64 per share, as compared to a net loss of \$51.2 million, or \$1.35 per share, for the three months ended September 30, 2021.

Cash and cash equivalents: As of September 30, 2022, the Company had cash and cash equivalents of \$34.3 million. The Company expects that the existing cash and cash equivalents, along with receipt of the \$50.0 million payment from Astellas and the \$25.6 million proceeds net of underwriting discounts, commissions and estimated offering expenses from the October 2022 public offering, will enable funding of operating expenses and capital requirements into the first quarter of 2024.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast with slides today at 8:00 am ET / 7:00 am CT to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 855-327-6837 (U.S./Canada) or 631-891-4304 (international). The conference ID for all callers is 10020611. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

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Forward-Looking Statements

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	\$ 16,391	\$ 39,528	\$ 77,308	\$ 94,025
General and administrative	8,683	11,153	30,019	29,518
Total operating expenses	25,074	50,681	107,327	123,543
Loss from operations	(25,074)	(50,681)	(107,327)	(123,543)
Other income (expense):				
Interest income	9	37	50	143
Interest expense	(1,241)	(543)	(3,002)	(737)
Other expense	(1)	-	(12)	-
Total other expense, net	(1,233)	(506)	(2,964)	(594)
Net loss	\$ (26,307)	\$ (51,187)	\$ (110,291)	\$ (124,137)
Net loss per common share, basic and diluted				
Net loss per common share, basic and diluted	\$ (0.64)	\$ (1.35)	\$ (2.77)	\$ (3.31)
Weighted average common shares outstanding, basic and diluted	40,937,808	38,003,954	39,761,764	37,495,537

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

	September 30, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 34,306	\$ 149,103
Prepaid expenses and other current assets	8,696	10,499
Total current assets	43,002	159,602
Restricted cash	2,637	2,637
Deferred lease asset	630	667
Property, plant and equipment, net	63,954	50,610
Other non-current assets	1,245	440
Total assets	\$ 111,468	\$ 213,956
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	\$ 15,521	\$ 21,763
Accrued expenses and other current liabilities	17,888	29,983
Total current liabilities	33,139	51,746
Build-to-suit lease liability	25,496	25,900
Term Loan, net	37,773	37,192
Other non-current liabilities	3,934	3,735

Total liabilities	100,342	118,573
<hr/>		
Stockholders' equity		
Preferred stock, \$0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of September 30, 2022 and December 31, 2021	-	-
Common stock, \$0.00001 par value per share; 200,000,000 shares authorized and 41,175,939 and 38,473,945 issued and outstanding as of September 30, 2022 and December 31, 2021, respectively	1	-
Additional paid-in capital	357,065	331,032
Accumulated deficit	(345,940)	(235,649)
Total stockholders' equity	11,126	95,383
Total liabilities and stockholders' equity	\$ 111,468	\$ 213,956

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies to Participate in JMP Securities Hematology and Oncology Summit

Nov. 29, 2022 4:01 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, Nov. 29, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced that it will participate in the JMP Securities Hematology and Oncology Summit, which is taking place virtually from Tuesday, December 6, 2022, through Wednesday, December 7, 2022. Taysha's President, Founder and Chief Executive Officer, RA Session II, will participate in a fire side chat with Silvan Turkan, Ph.D., Senior Research Analyst at JMP Securities, at 3:40 PM ET on Wednesday, December 7, 2022.

A webcast of the fire side chat will be available in the "Events & Media" section of the Taysha corporate website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 90 days.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Executive Leadership Changes

Dec. 16, 2022 6:18 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Chair of the Board of Directors, Sean P. Nolan, appointed Chief Executive Officer

Board Director, Sukumar Nagendran, M.D., appointed President and Head of R&D

DALLAS, Dec. 16, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, pivotal-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS) in both rare and large patient populations, today announced executive leadership changes effective immediately. Taysha's Chair of the Board of Directors, Sean P. Nolan, a highly experienced biopharmaceutical industry senior leader, has been appointed Chief Executive Officer, succeeding RA Session II, who has resigned from his operating role, but will continue to serve on the Company's Board of Directors. In addition, Sukumar (Suku) Nagendran, M.D., a Director on Taysha's Board of Directors, and an accomplished physician, drug developer, and biotech executive, has been appointed President and Head of R&D.

"I am excited to join the Company at such a dynamic time in our journey and energized to work with the team to expedite progress on our two lead clinical programs in Giant Axonal Neuropathy (GAN) and Rett syndrome, as well as further strengthen our strategic partnership with Astellas," said Mr. Nolan. "2023 is a crucial year for Taysha and it is imperative that we precisely execute as an organization on delivering key clinical and regulatory milestones as we endeavor to bring transformative therapies to patients and families suffering from devastating diseases."

Mr. Nolan continued, “On behalf of the entire Board, we thank RA for his many contributions since founding Taysha in 2019 and successfully guiding the Company through its seed and crossover funding, the initial public offering, advancing multiple programs into the clinic and bringing in the strategic investment from Astellas. He has been a valued partner that was foundational to furthering Taysha’s mission, and we wish him the best in his future endeavors.”

“Taysha has an industry leading pipeline, and I am thrilled to join the management team as we strive to have an enhanced impact on the development of potentially life changing treatments for monogenic diseases of the central nervous system,” said Dr. Nagendran. “I am excited about Taysha’s product candidates, its people, and the many opportunities ahead to help patients. I look forward to working more closely with Dr. Suyash Prasad, and the entire Taysha clinical team to further the advancement of our lead programs in GAN and Rett syndrome.”

Taysha anticipates hosting an investor call in mid-January once final minutes from the FDA Type B meeting on GAN are available to discuss feedback and next steps.

Mr. Nolan is an accomplished senior executive with over 30 years of biopharmaceutical experience. He previously served as Chief Executive Officer of the gene therapy company, AveXis Inc., until its acquisition by Novartis. While at AveXis ([AVXS](#)), Mr. Nolan led the company through an initial public offering and transitioned it into a fully integrated global organization with research, clinical, regulatory, manufacturing and commercial capabilities. He also previously served as Chief Business Officer of InterMune, Chief Commercial Officer of Reata Pharmaceuticals and Ovation Pharmaceuticals, and President of Lundbeck’s U.S. affiliate. Mr. Nolan currently serves as Executive Chairman of Jaguar Gene Therapy, and is a Board member of Encoded Therapeutics, Itsari Oncology, Taysha Gene Therapies, and Ventas. He holds a B.A. in Biology from John Carroll University.

Dr. Nagendran has more than 30 years of experience in key functional areas, including gene therapy development, clinical development strategy, medical affairs, and diagnostics. He previously served as Chief Medical Officer and President of R&D at Jaguar Gene Therapy. Prior to that, Dr. Nagendran was the Chief Medical Officer and Senior Vice President of AveXis Inc., a clinical-stage gene therapy company, from September 2015 to July 2018, prior to the company's acquisition by Novartis. At Quest Diagnostics, a provider of diagnostic information services, he served as Vice President of Medical Affairs from March 2013 to September 2015. Dr. Nagendran has also held key leadership positions at Pfizer, Novartis, Daiichi Sankyo, and Reata Pharmaceuticals. Prior to moving to the biotech industry, he practiced internal medicine, with a focus on diabetes and cardiovascular disease. Dr. Nagendran is a Mayo Alumni Laureate and founding member of the Robert Wood Johnson Legacy Society. He is also the sponsor for the Jerry Mendell award for Translational Science at the American Society of Gene and Cell Therapy which recognizes the extensive work required to bring gene and cell therapies to clinical trial, and the Fonseca-Nagendran Scholar award at the American Diabetes Association to enhance research in minority populations. Dr. Nagendran currently serves on the Board of Directors of SalioGen Therapeutics, Solid Biosciences, Cove, Medocity, Project Healthy Minds, and Taysha Gene Therapies. He holds an undergraduate degree in Biochemistry from Rutgers University and earned his M.D. from Rutgers Medical School, and trained in Internal Medicine at Mayo Clinic, Rochester.

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2022 GlobeNewswire, Inc.

Taysha Gene Therapies Provides Update on TSHA-120 Program in Giant Axonal Neuropathy and a 2023 Corporate Outlook

Jan. 31, 2023 4:01 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Type B end-of-Phase 2 meeting with U.S. Food and Drug Administration (FDA) provided additional clarity for TSHA-120 for the treatment of giant axonal neuropathy (GAN) ultra-rare disease program

- FDA acknowledged MFM32 as an acceptable endpoint with a recommendation to dose additional patients in a double-blind, placebo-controlled design to support Biologics License Application (BLA) submission

Organizational and business review by new management with operational, structural and personnel changes implemented to enhance execution

Dosing of first adult patient with Rett syndrome from ongoing trial in Canada expected in H1 2023; update of initial available clinical data anticipated in H1 2023 with quarterly updates primarily on safety thereafter

Submission of Clinical Trial Application (CTA) to United Kingdom (UK) MHRA for TSHA-102 in pediatric patients with Rett syndrome expected in mid-2023

Submission of an Investigational New Drug (IND) application for TSHA-102 for Rett syndrome to FDA planned in H2 2023

Conference call and live webcast today at 4:30 PM Eastern Time

DALLAS, Jan. 31, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a patient-centric, clinical -stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic rare diseases of the central nervous system (CNS), today provided an update on the TSHA-120 program in giant axonal neuropathy (GAN) and a corporate outlook for 2023.

"We expect to deliver on several key milestones in 2023, including the generation of first-in-human adult clinical data in Rett syndrome, CTA submission to MHRA to enable initiation of our pediatric Rett syndrome program and submission of an IND for Rett syndrome in the U.S. to further expand our clinical study footprint. For our GAN program, we received the formal FDA meeting minutes and recently submitted follow up questions to clarify some of their recommendations including the feasibility of a proposed study design and the totality of evidence required for BLA submission. Their feedback will help inform next steps for the program in this ultra-rare indication with no approved treatments," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "I believe that the operational, structural and personnel actions recently implemented position us well to execute across our near-term milestones and deliver on our commitments to key stakeholders, especially patients."

Clinical Program Updates

TSHA-120 in GAN:

- Receipt of formal written meeting minutes from FDA in January 2023 following completion of Type B end-of-Phase 2 meeting
 - Overall approach to manufacturing of pivotal/to-be marketed product deemed appropriate pending review of a planned submission of Chemistry, Manufacturing, and Controls (CMC) data package for TSHA-120
 - FDA acknowledged MFM32 as an acceptable endpoint with a recommendation to dose additional patients in a double-blind, placebo-controlled design to support BLA submission
- Awaiting response from FDA on follow up questions the Company submitted on recommended design and totality of evidence required for BLA submission

TSHA-102 in Rett syndrome:

- Dosing of the first adult patient with Rett syndrome anticipated in H1 2023
- Initial available clinical data for TSHA-102 in the adult Rett syndrome study expected in H1 2023 with planned quarterly updates on available clinical data primarily on safety from the adult study thereafter

- Company anticipates submission of a CTA to UK MHRA for TSHA-102 in pediatric patients with Rett syndrome in mid-2023
- Company plans to submit an IND application for Rett syndrome to FDA in H2 2023

Corporate Updates

- Operational, structural and personnel changes implemented following thorough business review to enhance execution

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 4:30 pm ET to provide regulatory feedback from FDA on the GAN program and a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13736009. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, such as TSHA-120 and TSHA-102 and including our preclinical product candidates, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, the potential market opportunity for these product candidates, our corporate growth plans and the impacts of our corporate operational, structural and personnel changes. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2021 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, both of which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

Company Contact:

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Chief Corporate Affairs Officer

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

[Home](#) > [Stock Ideas](#) > [Quick Picks & Lists](#)

Top 10 Stocks For 2024

Dec. 31, 2023 10:00 AM ET | 93 Comments | 110 Likes



Steven Cress

SA Quant Strategist

Summary

- As of December 31st, 2023, SA's Top 10 Quant Picks outperformed the S&P 500 by ~42.5%, with top picks including (SMCI) up 242%, (MOD) +196%, (MNSO) + 85%, and (PDD) +71%.
- SA's Quant strategy identifies stocks collectively strong on Growth, Value, Profitability, Earnings Revisions, and Momentum.
- Many financial pundits expect inflation data and economic demand to soften in 2024. Investors may gain and find opportunities with a good investment strategy despite conflicting outlooks.
- As we enter 2024 amid divided economic and financial predictions, consider SA's Quant Rating System, which offers powerful analytics and draws on a model whose Top 10 stock recommendations crushed the S&P 500 in 2023.
- I am Steven Cress, Head of Quantitative Strategies at Seeking Alpha. I manage the quant ratings and factor grades on stocks and ETFs in Seeking Alpha Premium. I also lead [Alpha Picks](#), which selects the two most attractive stocks to buy each month, and also determines when to sell them.



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The general consensus was anticipation of a recession in 2023 and that equity markets would struggle as bonds rallied. Picking the best stocks to start the New Year can take time and effort, especially when analysts are divided about the outlook. At the start of 2023, our top selections experienced some turbulence. However, as the year progressed and investors gravitated towards stocks with solid fundamentals, the returns from these stocks were quite rewarding.

My Top 10 picks for 2023 crushed performance of the S&P 500

Symbol	Price	Change	Change %	Shares	Cost	Today's Gain	Today's % Gain	Total Change	Total % Change	Value
ASC	14.09	-0.02	-0.14%	69.44	14.40	-1.39	-0.14%	-21.53	-2.15%	978.41
ENGIY	17.60	-0.04	-0.23%	69	14.48	-2.76	-0.23%	215.28	21.55%	1,214.40
HDSN	13.49	0.13	0.97%	97.80	10.22	12.71	0.97%	319.81	32.00%	1,319.32
JXN	51.20	-0.74	-1.42%	28.30	35.29	-20.94	-1.42%	450.25	45.08%	1,448.96
MNSO	20.40	0.16	0.79%	90	11.00	14.40	0.79%	846.00	85.45%	1,836.00
MOD	59.70	-1.01	-1.66%	49.67	20.13	-50.17	-1.66%	1,965.44	196.57%	2,965.30
PDD	146.31	0.96	0.66%	11.69	85.51	11.22	0.66%	710.75	71.10%	1,710.36
SMCI	284.26	-7.19	-2.47%	11.92	83.08	-85.70	-2.47%	2,398.07	242.15%	3,388.38
VLO	130.00	-0.09	-0.07%	7.95	125.75	-0.72	-0.07%	33.79	3.38%	1,033.50
VRNA	19.88	-0.28	-1.39%	37.80	26.44	-10.58	-1.39%	-247.97	-24.81%	751.46
TOTAL						-133.93	-0.80%	6,669.89	66.86%	16,646.10

My Top 10 picks for 2023 crushed performance (SA Premium)

In 2024, many investors expect inflation data and economic demand to soften, potentially heightening recession fears. Despite conflicting outlooks for 2024, I believe a sound investment strategy offers the potential for continued gains. For investors optimistic about the future, should we enter a contractionary period filled with market turmoil, consider identifying high-quality stocks with strong fundamentals based on valuation, growth, profitability, momentum, and EPS Revisions.

My Top 10 picks for 2024 have also been outperforming the S&P 500 in 2023

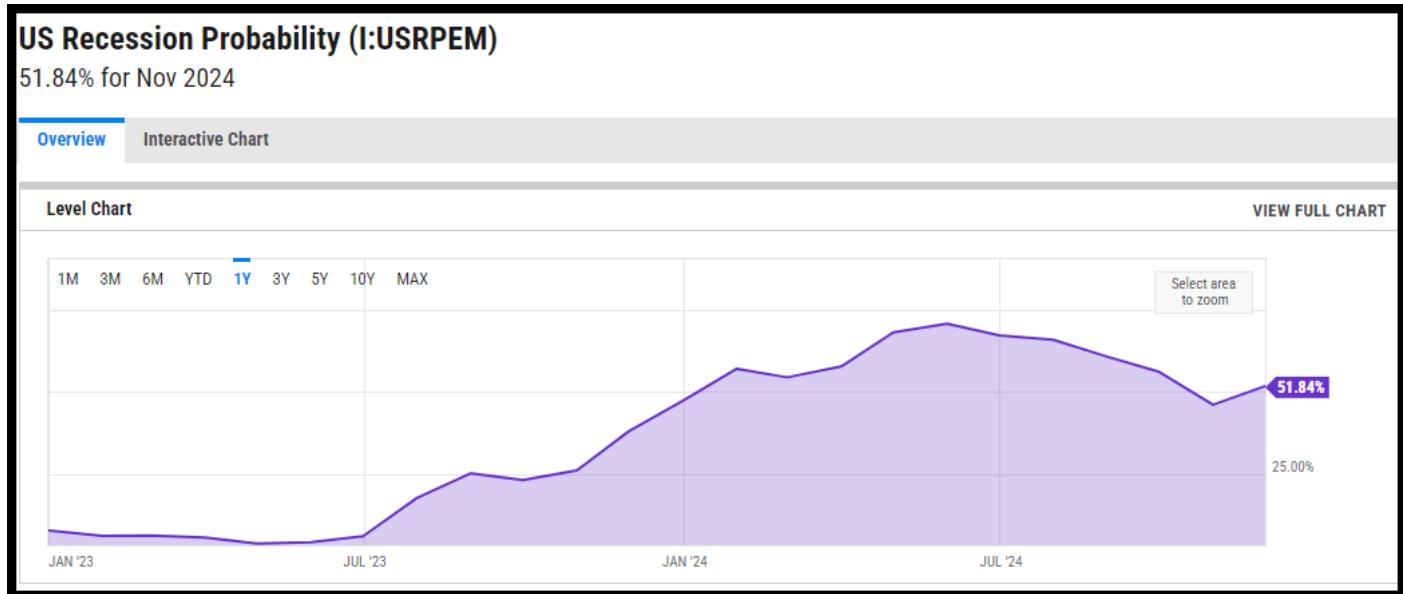


My Top 10 picks for 2024 are outperforming the S&P 500 (as of 12/29/23) (Trading View, Seeking Alpha)

On November 1st, the Fed [signaled](#) a likely end to its hiking cycle, prompting a rally with the major indexes up double-digits. Through the last trading day of the year, my Top 10 Stocks for 2023 crushed performance of the S&P 500, +66.86% vs. the S&P 500 +24.2%. So, to kick off the new year, I have ten fresh stock picks for 2024.

Will there be a recession in 2024?

The U.S. economy was resilient throughout 2023 and avoided a technical recession. Stable labor markets and the Fed pausing rate hikes have created some optimism for the new year as the markets experienced a turnaround from the crushing blows of 2022. While some analysts anticipate a softer landing, inflation, elevated interest rates, slowing GDP growth, and potential economic and cyclical shifts in the first half of 2024 pose risks that could result in a recession.



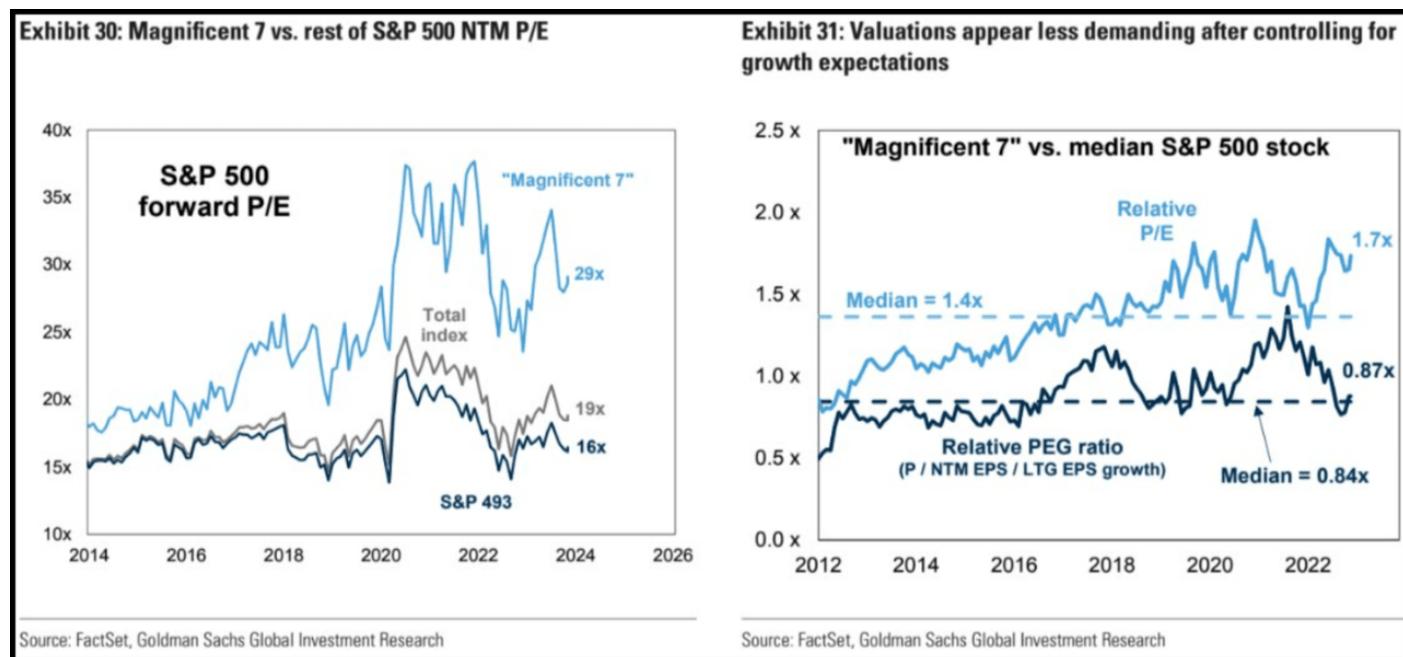
Mortgage, credit card, and auto loan delinquency rates have risen. The 10-year and two-year Treasury yield curve has continued to be inverted since 2022, a historically strong recession indicator, as Fed projections indicate that growth will slow down to 1.4% in 2024. Although U.S. GDP grew 5.2% in Q3 amid rising inflation and interest rates, it remains to be seen if 2024 will avoid a recession. The key is focusing on stocks best equipped to withstand market volatility. Quant Strong Buy-rated stocks in varied sectors and industries offer diversification that can help minimize risk, maximize returns, and weather unexpected volatility in the coming New Year.

A Rotation Out Of the Magnificent 7?

During 2023, seven stocks, known as the Magnificent 7, drove the performance of the S&P 500. Among the most prominent technological names, the Magnificent 7 are the largest market cap stocks in the S&P 500. Driven by high-end software and hardware, cloud computing, a reversion from poor stock performance in 2022, and the AI boom, these seven megatech names led the charge in 2023.

In 2022, the performance narrative for the Magnificent 7 was completely different from the performance that transpired in 2023. Post-pandemic, these stocks experienced a sharp sell-off. Investors, fearing a severe recession, liquidated significant positions to generate cash. However, in 2023, with clearer insights into inflation and unfolding global conflicts, and following a year with \$12 trillion in wealth wiped out and Big Tech accounting for over \$4 trillion in market cap losses, investors sought safety, leading to increased inflows into mega tech stocks.

The Magnificent seven appear highly overvalued compared to the total index



The Magnificent seven is highly overvalued compared to the total index (FactSet, Goldman Sachs Global)

Looking at the trailing and forward P/E value of the Magnificent 7 compared to the rest of the market, I view these Big Tech constituents as highly overvalued. The overvalued state for the largest market cap stocks bodes well for a potential **rotation**, as investors typically seek stocks with solid growth and fair valuations in a declining rate environment. A rotation into fundamentally strong stocks with **strong momentum** is why Seeking Alpha's Quant system has identified ten stocks for 2024.

Top Consumer Discretionary Stocks

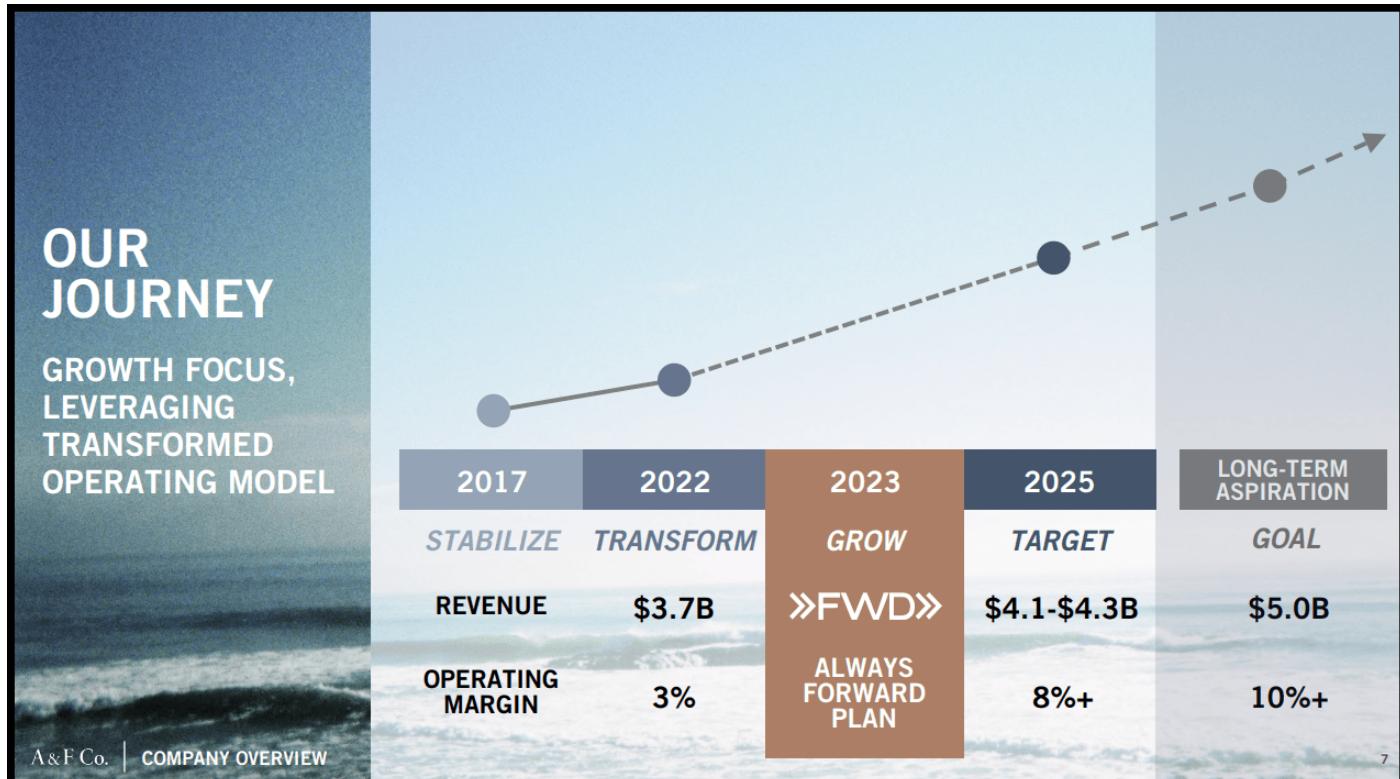
The consumer discretionary sector offers an array of industries, so using top Quant picks, I've selected four stocks ranking among the top in their industry. The consumer discretionary sector's ([XLY](#)) performance for 2023 was strong despite some economic slowing and inflation prompting many consumers to budget. In 2023, XLY was a top-performing sector, +39% behind Tech ([XLK](#)), +55%, and Communications ([XLC](#)) sectors, +52% for the year. Although periods of downturn and recession can negatively impact stocks, the much-anticipated recession of 2023 was averted. With economists' outlook for 2024 mixed and recession fears back on the table, I [screened](#) for 10 top Quant-rated stocks, and the picks below may offer upside opportunities for the new year.

1. Abercrombie & Fitch Co. ([ANF](#))

- Market Capitalization: \$4.54B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 1 out of 533
- Quant Industry Ranking (as of 12/29/23): 1 out of 41

Record sales, a strong management team, and rebranding have led the turnaround for all-American [retailer](#) Abercrombie & Fitch Co. ([ANF](#)). Rallying +293% over the last year, ANF has consecutively crushed earnings, delivering top-line total net sales of \$935M in Q2, up 16% from last year, and Q3 net sales of \$1.06, up 20%. Meeting the needs of its customers through digital transformation and embracing the online and e-commerce experience, ANF is showcasing the company's ability to accelerate sales growth and profitability.

ANF Stock is focused on growth for the future



ANF Stock is focused on growth for the future (ANF Q2 2023 Investor Presentation)

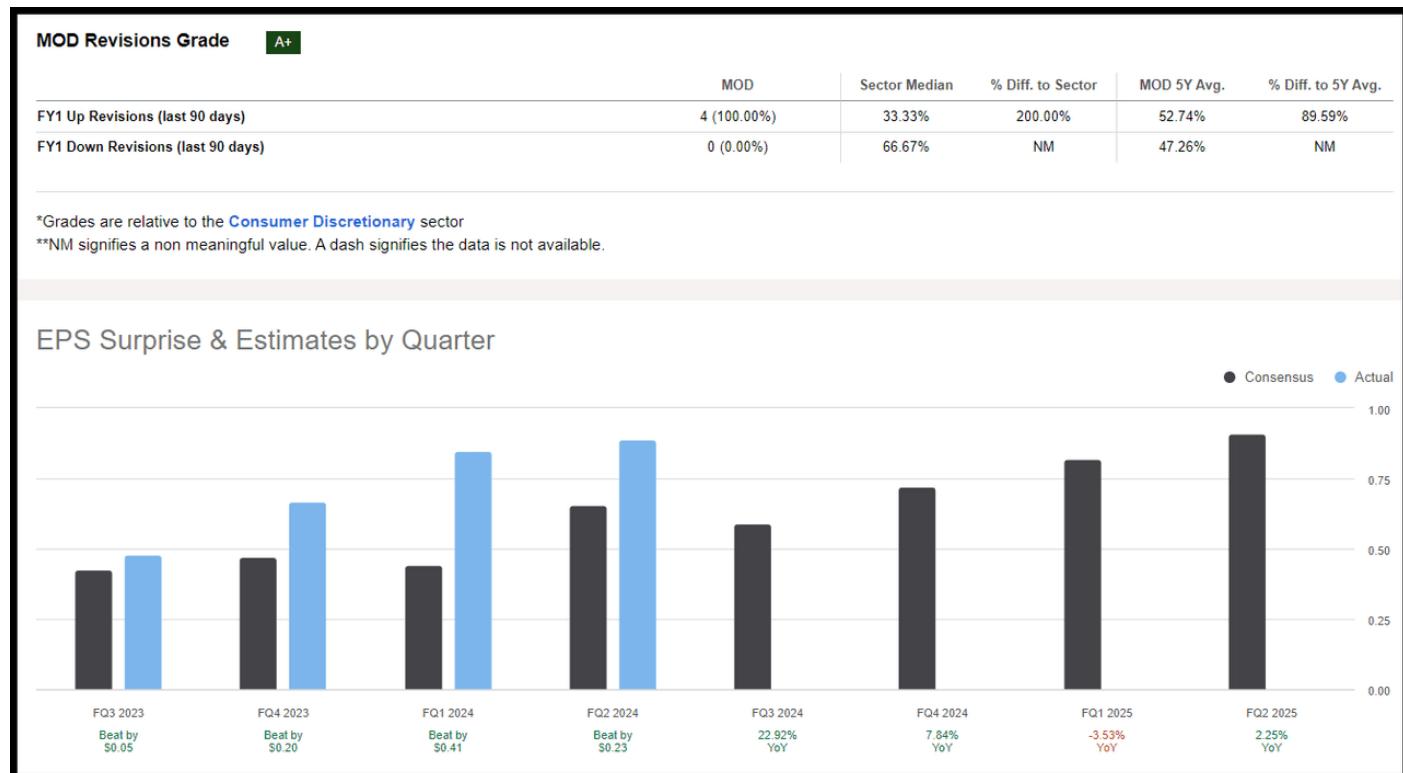
Abercrombie's Q3 EPS of \$1.83 beat by \$0.65, and it continues to expand margins. In addition to its brand diversification and international footprint, ANF's Quant ratings are exceptional, so much so that it was introduced to the [Alpha Picks](#) portfolio on October 2, 2023, and has returned +55% and doubled its market cap. Despite its run-up, the stock continues to trade at a [discount](#), highlighted by a forward P/E ratio of 15.87x versus the sector median of 17.88x. Abercrombie's trailing PEG ratio is a -92.88% difference to the sector, and increasing free cash flow, a strong balance sheet, and positive momentum into 2024 are a few of the many factors into why Abercrombie & Fitch is currently the top-ranked stock in its sector and industry.

2. Modine Manufacturing Company ([MOD](#))

- Market Capitalization: \$3.17B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 5 out of 533
- Quant Industry Ranking (as of 12/29/23): 1 out of 35

Modine Manufacturing ([MOD](#))'s legacy as a top player in auto parts and equipment and capitalizing on heating, ventilation, and air conditioning (HVAC) features are significant drivers of their business. Leveraging tech advancements and improvised energy efficiency to aid the rising awareness and sustainability contributes to why MOD is currently [#1 Quant-ranked](#) in its industry.

MOD Stock consecutively beats earnings



MOD Stock consecutively beats earnings (SA Premium)

Although EVs have lost some of their [buzz](#), MOD has benefitted from the electric vehicle trend and possesses one of the largest markets in the U.S. and Europe. Offering commercial electric vehicle parts, refrigeration, and original equipment manufacturer (OEM) parts, MOD's investment in developing EV-focused segments has aided its expanding margins and stock price. MOD is +195% over the last year and surged past earnings expectations with Q3 EPS of \$0.89 beating by \$0.23, and revenue of \$620.50M beating by \$4.33M. Adjusted EBITDA of \$81.2 saw an increase of 59% from the previous year, and the EBITDA margin was +13.1% from Q3 of 2022. These results prompted four analysts to revise estimates up over the last 90 days for an A+ Revisions Grade. Although the stock is trading near its 52-week high and comes at a relative premium given its D+ [Valuation grade](#), MOD's trailing P/E ratio of 15.68x is a 14% difference to the sector, and its' trailing PEG of 0.23x versus the sector's 0.56x is more than a 58% difference. With bullish momentum, tremendous growth, and profitability, consider MOD for a portfolio, although some prudence is needed if entering at the current price point.

3. M/I Homes, Inc. ([MHO](#))

- Market Capitalization: \$3.87B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 4 out of 533
- Quant Industry Ranking (as of 12/29/23): 1 out of 23

Despite volatility in a rapidly changing real estate market as many homebuilders experience declines, M/I Homes, Inc. ([MHO](#)) has soared. Engaging in the construction and sale of single-family homes in the U.S., MHO has rallied +198% over the last year and is trading near its 52-week high of \$140.73. Despite the stock's surge, MHO's B+ [Valuation Grade](#) highlights its discounted price, and when you couple it with the inventory shortages and demand for housing, buying MHO at its current level can be very attractive.

MHO stock trades at an attractive discount

MHO Valuation Grade	B+	Sector Relative Grade	MHO	Sector Median	% Diff. to Sector
P/E Non-GAAP (TTM)	A	7.83	14.72	-46.81%	
P/E Non-GAAP (FWD)	A	7.93	16.27	-51.28%	
P/E GAAP (TTM)	A	8.06	18.24	-55.80%	
P/E GAAP (FWD)	A+	7.93	17.88	-55.67%	
PEG GAAP (TTM)	D	1.61	0.56	189.00%	
PEG Non-GAAP (FWD)	-	-	1.66	-	
EV / Sales (TTM)	B	0.95	1.24	-23.08%	
EV / Sales (FWD)	B	0.96	1.26	-23.45%	
EV / EBITDA (TTM)	A	6.33	11.19	-43.41%	
EV / EBITDA (FWD)	-	-	10.17	-	
EV / EBIT (TTM)	A+	6.48	15.27	-57.58%	
EV / EBIT (FWD)	A+	6.39	14.52	-55.95%	

MHO stock trades at an attractive discount (SA Premium)

MHO's forward P/E ratio of 7.93x and forward EV/EBIT trade more than a -55% difference to the sector. Through innovation and demographic trends, MHO has capitalized by creating a [Smart Series](#) affordable home that appeals to Millennials and has an average selling price of \$481,000. This price point has generated higher margins and better return on investment for MHO while attempting to solve the home affordability problem. Boasting record revenues, MHO's third quarter generated \$1.05B, a 3% increase, and EPS of \$4.82, beat by \$0.55. In a strong financial position, MHO maintains a backlog of inventory, continues to write new contracts, and is [buying back](#) shares of its stock. Highlighted by President & CEO Robert Schottenstein, MHO [logged](#) "one of the best [quarters] in company history, highlighted by record revenue, record income, a 50% increase in new contracts, and very strong margins and returns." Embracing technology, innovation, and looking toward the future, MHO is not the only quant Strong Buy-rated stock on cloud nine.

4. GigaCloud Technology Inc. ([GCT](#))

- Market Capitalization: \$737.32M
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 1 out of 533

- Quant Industry Ranking (as of 12/29/23): 1 out of 6

GigaCloud Technology Inc. ([GCT](#)), a leading business-to-business market provider, combines technology and years of marketplace industry experience to connect manufacturers (primarily in Asia) with resellers worldwide. Delivering large parcels and e-commerce around the world, according to [Nasdaq.com](#), GCT is one of the [best stocks](#) to buy now in terms of quality, with the highest return on capital.

GigaCloud Technology has exceptional Quant Ratings & Factor Grades

Ratings Summary

SA Analysts	BUY	4.00
Wall Street	STRONG BUY	4.66
Quant	STRONG BUY	4.99

Factor Grades

	Now	3M ago	6M ago
Valuation	B	A	-
Growth	A-	B+	-
Profitability	A-	B+	-
Momentum	A+	B+	-
Revisions	A-	A+	-

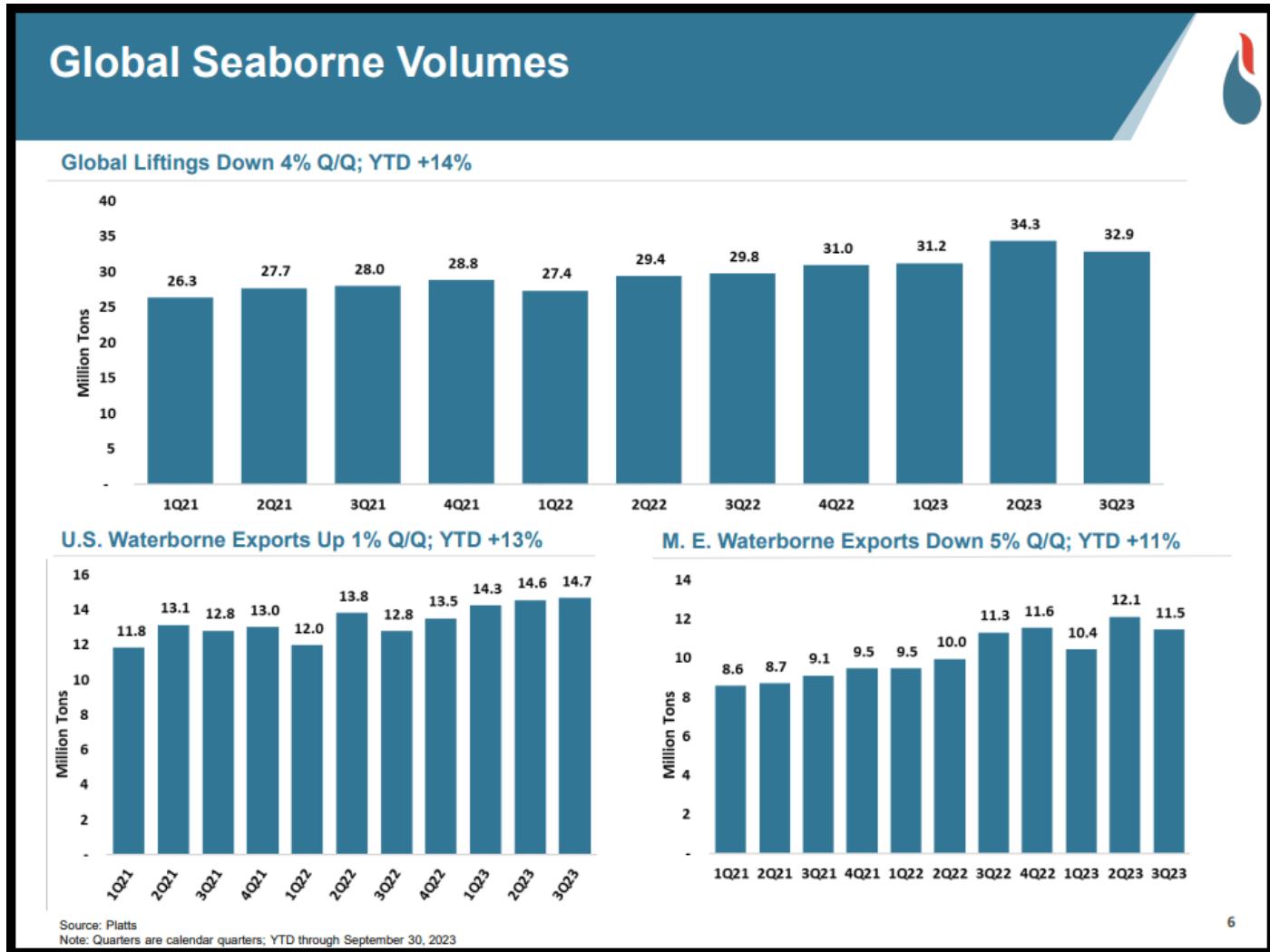
GigaCloud Technology has exceptional Quant Ratings & Factor Grades (SA Premium)

Factor Grades rate investment characteristics on a sector-relative basis, and GCT showcases some of the best. In addition to SA's Quant Ratings, Wall Street and Seeking Alpha Analysts agree that GCT is a buy. Q3 adjusted EBITDA saw a 150% increase, marking the third consecutive quarter of record profitability. GCT has fueled its growth to meet consumer demands through strategic partnerships, [acquisitions](#), and product diversification. Completing its acquisition of [Noble House](#) for \$85M in cash, GigaCloud will add over 8,000 SKUs and a strong supply chain system to add depth to its already diverse product offerings and business. GigaCloud's Q3 EPS of \$0.59 beat by \$0.24, and revenue of \$178.17M beat by nearly 40% year-over-year. Building on its current fulfillment and logistics capabilities, GigaCloud is up 226% over the last year. At an extreme discount, GCT's trailing PEG is a 99% difference from the sector, and a forward P/E of 9.16x versus the sector's 17.88x indicates the stock is ripe for addition to a portfolio. Consider this discounted stock ready for growth, along with my next picks, an energy and industrial stock.

5. Dorian LPG Ltd. ([LPG](#))

- Market Capitalization: \$1.80B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 1 out of 234
- Quant Industry Ranking (as of 12/29/23): 1 out of 55

One of the [top-performing](#) energy stocks of 2023, Dorian LPG Ltd. ([LPG](#)), together with its subsidiaries, owns and operates 25 Very Large Gas Carriers ((VLGCs)), used for oil and gas storage and transportation. Despite geopolitical and trade tensions around the globe, Dorian's modern fleet, tech-advanced vessels, and focus on environmental regulations and energy transition have helped LPG grow.



6

Dorian Stock Seaborne Volumes (Dorian Stock Seaborne Volumes (Dorian LPG Q2 Investor Presentation))

Despite missing Q2 earnings, analysts are revising estimates up as global seaborne volumes have seen increases, and overarchingly, Dorian experienced a strong quarter with record EBITDA, allowing the Board to declare another \$1 per share [dividend](#).

"The strength of our balance sheet with net debt to total capitalization of about 30% and attractive financing conditions enabled us to return about \$650 million to shareholders since our IPO while also pursuing a conservative fleet renewal policy and continuing to invest in fleet operational efficiencies and decarbonization initiatives," said John Hadjipateras, Dorian President & CEO.

Dorian has a solid outlook, possessing a strong free cash flow balance and capitalizing on its second-best TCE utilization of 96.5%, which yielded an adjusted \$62,818. LPG trades at a discount, highlighted by a B overall [Valuation Grade](#) and some "game-changing" factors.

LPG Stock trades at an attractive valuation

LPG Valuation Grade	B	Sector Relative Grade	LPG	Sector Median	% Diff. to Sector
P/E Non-GAAP (TTM)	B		7.04	8.78	-19.84%
P/E Non-GAAP (FWD)	A-		5.37	10.08	-46.75%
P/E GAAP (TTM)	B		6.98	9.09	-23.20%
P/E GAAP (FWD)	A-		5.29	10.49	-49.55%
PEG GAAP (TTM)	B+		0.04	0.17	-74.88%

LPG Stock trades at an attractive valuation (SA Premium)

Jefferies increased its price target from \$37 to \$50 and upgraded the stock from Hold to Buy, [citing](#) that the latest [Panama Canal](#) restrictions could change the outlook for spot and charter rates, with LPG benefitting from a “game-changer in an already tight VLGC market.”

6. Rolls-Royce Holdings, plc ([OTCPK:RYCEY](#))

- Market Capitalization: \$31.74B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 6 out of 657
- Quant Industry Ranking (as of 12/29/23): 1 out of 59

Major conflicts in 2023 prompted investors to seek safe haven, and as they continue into 2024 along with talks of recession, defensive stocks were among some of the top gainers in Q4 of 2023.

In November, I wrote about Rolls-Royce Holdings plc ([OTCPK:RYCEY](#)) in an [article](#) titled Top Defensive Stocks for Turbulent Times. RYCEY is versatile and offers innovation as an operator of industrial technology. This former parent company of luxury car manufacturer Rolls-Royce is an aero-engine manufacturer that operates four segments: Civil Aerospace, Defense, Power Systems, and New Markets. RYCEY's fundamentals and a legacy of products and services since 1884 have enabled it to thrive despite the turbulent times.

Rolls-Royce Rallies High

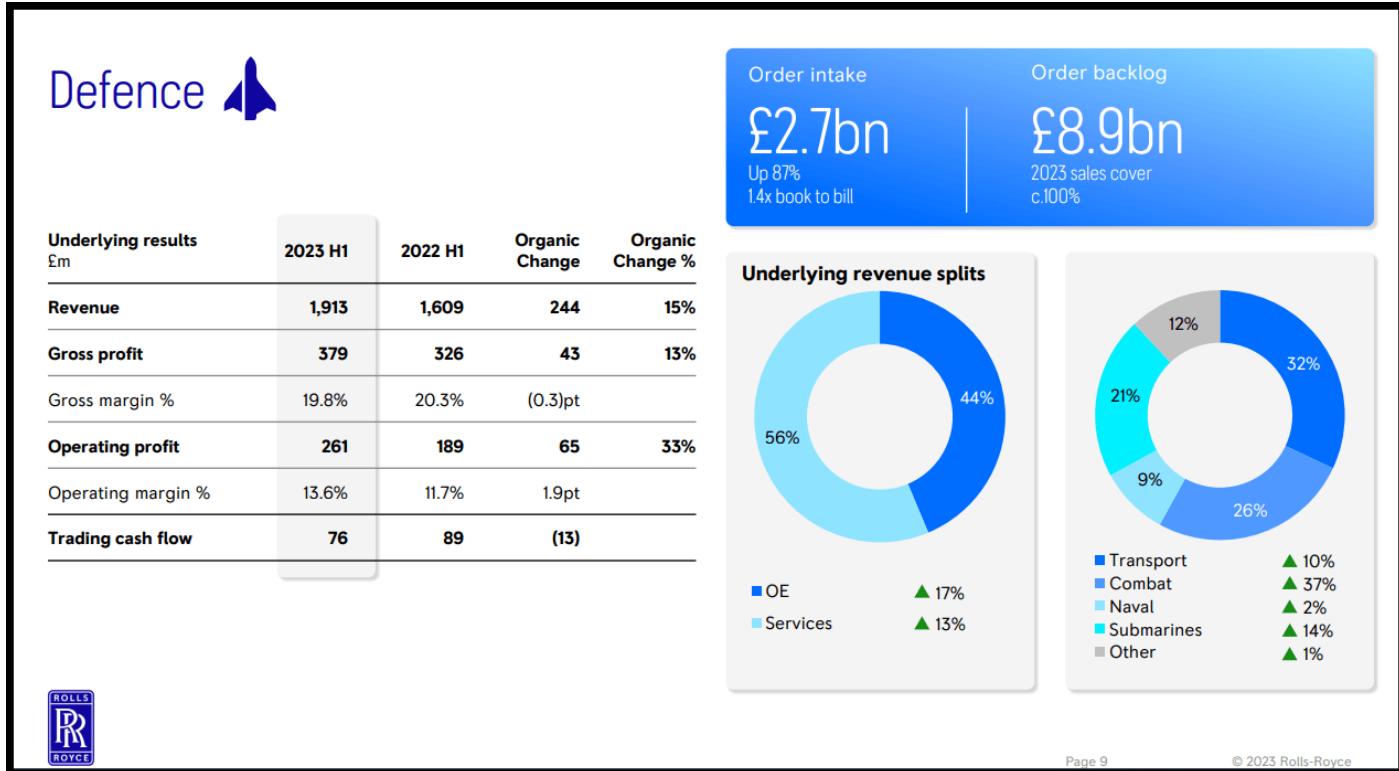


Rolls-Royce Rallies High (SA Premium)

Rolls-Royce's trailing P/E GAAP ratio is 15.16x compared to the sector median's 23.32x, and its forward PEG ratio is more than a 73% difference to the sector despite the stock trading near its 52-week high.

Up 227% to end the year, RYCEY hit a four-year high, forecasting a return on capital of 18%. CEO Tufan Erginbilgic said during a [conference call](#), “*We’re looking to recreate a new Rolls-Royce which is high performing, competitive, resilient, and a growing company. We’ll unlock our full potential as we translate engineering excellence into strong financial performance.*”

Defense is RYCEY's second-largest segment, with consistent government and defense contracts revenue. Increasing orders, margin improvements led by civil and defense, and strong financials have resulted in the Defense division being up 87% for the first half of the year, with 1.4x book to bill and a tremendous order backlog to support the company's future growth.



RYCEY Stock's Defense Segment (RYCEY Investor Presentation)

Defend your portfolio with Rolls-Royce in the new year, which offers a defensive investment amid global turbulence.

Top Financial Stock

Notwithstanding the 2023 banking crisis, financials tend to benefit most from elevated rates and high inflation, so I've selected a top financial for 2024.

7. Intesa Sanpaolo S.p.A. (OTCPK:ISNPY)

- Market Capitalization: \$53.34B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 4 out of 695
- Quant Industry Ranking (as of 12/29/23): 1 out of 67

One of the largest Eurozone banking groups, Intesa Sanpaolo ([OTCPK:ISNPY](#)), is an Italy-based financial company offering various financial products and services through six segments, including wealth management and insurance. Currently, ISNPY is Quant-Ranked the #1 [Diversified Bank Stock](#) in its industry and is trading at a steep discount, showcased by its A+ [Valuation Grade](#), which includes a PEG ratio that's an 85% difference to the sector.

ISNPY Valuation Grade	A+	Sector Relative Grade	ISNPY	Sector Median	% Diff. to Sector
P/E Non-GAAP (TTM)	A-	A-	8.05	10.25	-21.48%
P/E Non-GAAP (FWD)	A	A	6.85	10.80	-36.62%
P/E GAAP (TTM)	A	A	7.20	11.05	-34.86%
P/E GAAP (FWD)	A+	A+	5.80	11.25	-48.51%
PEG GAAP (TTM)	A	A	0.06	0.40	-84.76%
PEG Non-GAAP (FWD)	A+	A+	0.22	1.41	-84.72%

ISNPY Stock Valuation Grade (SA Premium)

Although the stock has experienced some volatility, its fundamentals are excellent, and overall performance has maintained its Strong Buy Quant rating for most of 2023, going into 2024. ISNPY is +30% through year-end with solid momentum and crushed Q3 earnings.

EPS of \$0.76 beat by \$0.15 and revenue of \$6.83B beat by +36% year-over-year. ISNPY's Q3 delivered the best net income, best-ever operating income, and best operating margin in 16 years, resulting in improved guidance. During the Q3 Earnings Call, CEO Carlo Messina stated:

"Looking ahead, 2024 and 2025 net income will be higher than in 2023. Our dividend yield is the highest in Europe at 11.5%. In the first nine-months, we accrued cash dividends of €4.3 billion and completed the €1.7 billion buyback. In a few weeks, we will pay an interim dividend of €2.6 billion, that means a dividend per share of €0.144 almost doubling the interim dividend of last year."

Focused on client relationships and service excellence, ISNPY strives to strengthen its presence globally, improve customer service and satisfaction, and innovate through technology.

Technology and Communications Stocks

Technology and communication stocks have led the pack in 2023. Given that Big Techs have led performance, I'm offering one Mega-tech and two under-the-radar tech stocks for a potential [rotation](#) out of the sector, with Quant Strong-Buy ratings worth considering for a portfolio.

8. Meta Platforms, Inc. ([META](#))

- Market Capitalization: \$920.83B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 1 out of 245
- Quant Industry Ranking (as of 12/29/23): 1 out of 60

Meta Platforms Inc. ([META](#)) is a popular communications and innovative company that allows people to connect and share with friends. Significantly focused on AI and virtual reality, META develops products that allow users to connect and share with friends. Strong fundamentals, growth, tremendous profitability, and continued technological advancements allow it to rally. Up 194% over the last year, its strong user base, monetization of Instagram and WhatsApp, and e-commerce integration have been growth drivers. META has beaten earnings consecutively, with the latest Q3 EPS of \$4.39, beating by \$0.79, and revenue of \$34.15B, beating by +23% year-over-year.

META Profitability Grade	A+	Sector Relative Grade	META	Sector Median	% Diff. to Sector
Gross Profit Margin (TTM)	A		80.12%	48.90%	63.82%
EBIT Margin (TTM)	A+		34.41%	8.08%	325.90%
EBITDA Margin (TTM)	A+		42.58%	18.88%	125.50%
Net Income Margin (TTM)	A		23.42%	3.21%	628.96%
Levered FCF Margin (TTM)	A		23.19%	7.65%	203.04%
Return on Common Equity (TTM)	A		22.28%	3.41%	553.97%
Return on Total Capital (TTM)	A+		16.53%	3.55%	365.11%
Return on Total Assets (TTM)	A+		13.75%	1.24%	1,009.70%
CAPEX / Sales (TTM)	A+		22.56%	4.09%	452.12%
Asset Turnover Ratio (TTM)	B		0.64x	0.52x	24.73%
Cash From Operations (TTM)	A+		\$66.22B	272.33M	24,215.73%
Cash Per Share (TTM)	A+		14.35	1.57	816.06%
Net Income Per Employee (TTM)	A+		449.26K	6,894.82	6,415.85%

*Grades are relative to the [Communication Services](#) sector

META Stock Profitability Grades (SA Premium)

Although the stock is trading at a premium with a 'D' [Valuation Grade](#), its forward PEG of 1.25x compared to the sector's 1.56x is a -19.90% difference to the sector. Given its significant cash from operations of over \$66B, tremendous growth, and profitability grades, consider this stock for a portfolio as it continues to innovate, facilitate e-commerce activities, and invest heavily in AI.

9. Celestica Inc. ([CLS](#))

- Market Capitalization: \$3.52B
- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 4 out of 552
- Quant Industry Ranking (as of 12/29/23): 1 out of 18

A leader in global supply chain solutions, Celestica Inc. ([CLS](#)) is a top [electronic manufacturing services](#) (EMS) company focused on technology and capitalizing on AI trends. Trading at an extreme discount, CLS' Valuation Grade is an A-, showcasing stellar underlying metrics that include a forward P/E ratio of 15.66x versus the sector median of 29.33x, and forward PEG of 0.67x, a 69% difference to the sector.

CLS Valuation Grade	A-	Sector Relative Grade	CLS	Sector Median	% Diff. to Sector
P/E Non-GAAP (TTM)	A-	A-	13.27	22.48	-40.97%
P/E Non-GAAP (FWD)	A	A	12.56	25.10	-49.96%
P/E GAAP (TTM)	A-	A-	17.64	27.56	-35.99%
P/E GAAP (FWD)	A	A	15.66	29.33	-46.62%
PEG GAAP (TTM)	B+	B+	0.32	1.14	-72.10%
PEG Non-GAAP (FWD)	A+	A+	0.67	2.17	-69.02%
EV / Sales (TTM)	A+	A+	0.51	3.18	-84.10%
EV / Sales (FWD)	A+	A+	0.50	3.00	-83.27%
EV / EBITDA (TTM)	A	A	7.30	16.99	-57.01%
EV / EBITDA (FWD)	A	A	7.05	16.30	-56.73%
EV / EBIT (TTM)	A	A	9.29	21.86	-57.52%
EV / EBIT (FWD)	A	A	9.10	21.00	-56.69%
Price / Sales (TTM)	A	A	0.46	3.15	-85.53%
Price / Sales (FWD)	A	A	0.45	3.08	-85.53%

CLS Stock Valuation Grades (SA Premium)

A multinational electronics manufacturer with diversified services, CLS is currently ranked #1 in its [industry](#) given its range of diversified offerings focused on advanced technologies, supply chain optimization, being at the forefront of tech advancements in its industry, and having a global presence. With the ability to adapt to industry trends and maintain a strong network and relationship, CLS has increased margins and consecutively crushed earnings, with Q3 EPS of \$ 0.65 that beat by \$0.05 and revenue of \$2.04B beating by \$52.78M. Although the stock is trading near its 52-week high, CLS' ability to maintain supply chain flexibility and resilience and adapt to industry trends while managing costs contributes to its bullish momentum. Consider this stock for a 2024 portfolio, along with my last pick,

10. AppLovin Corporation ([APP](#))

- Market Capitalization: \$13.69B

- Quant Rating: Strong Buy
- Quant Sector Ranking (as of 12/29/23): 7 out of 552
- Quant Industry Ranking (as of 12/29/23): 1 out of 191

Gamers love AppLovin Corporation ([APP](#)), which has a diverse gaming portfolio focused on application software so that app developers and mobile gamers can enhance their marketing for business growth. Focused on the user experience within apps to attract and retain users and developers, AppLovin offers end-to-end software solutions, leveraging AI for data-driven marketing decisions. Unique tools that allow developers to increase user engagement for revenue generation and the stock's portfolio of free games and applications coupled with an aggressive acquisition strategy have led to its +\$880M cash from operations.

APP Momentum Grade	A+	Sector Relative Grade	APP	Sector Median	% Diff. to Sector
3M Price Performance		C-	2.57%	13.94%	-81.59%
6M Price Performance		A+	58.54%	4.65%	1,159.43%
9M Price Performance		A+	191.77%	12.26%	1,464.04%
1Y Price Performance		A+	333.62%	25.97%	1,184.64%

*Grades are relative to the [Information Technology](#) sector

APP Stock Momentum Grade (SA Premium)

AppLovin has experienced tremendous growth, including a stellar one-year price performance, significantly outperforming the sector median peers quarterly. Although the APP is currently trading at a relative [premium](#), its all-important forward PEG ratio of 0.64x versus the sector's 2.17x is a -70% difference to the sector. AppLovin continues to gain market share, as showcased by consecutive earnings beats. In addition to 14 analysts revising estimates up over the last 90 days, APP's Q3 EPS of 0.30 beat by \$0.03, and revenue of \$864.26M beat by \$21.20% year-over-year. APPLovin has repurchased \$1.2B in Class A common stock through Q3, paid down \$250M in term loans, and continues to improve its balance sheet. Focused on the user experience and global expansion, AppLovin is a stock to love, along with each of the Quant Strong Buy-rated stocks for 2024.

Celebrate 2024 with 10 Top Quant-Rated Stocks

Analysts' and economists' outlooks about what 2024 will bring are mixed. Will the Fed continue its rate hikes? Will there be a Fed-forced recession, slowing growth, and how will equities fare into the New Year? While past performance is no guarantee of the future, Seeking Alpha's [portfolio](#) of Top 10 Stocks for 2023 delivered incredible results, Up ~67% compared to the S&P 500's +24.2%, a testament to our Quant Ratings. Stocks with tremendous fundamentals should benefit in the long term. Each of my 2024 recommended stocks offers the potential for double-digit positive performance and has delivered incredible results amid the headwinds in 2023.

Although this year, the markets were carried by seven of the biggest names in technology, [Top Technology stocks](#) are attractive, but we could see a rotation into smaller companies. Although many of my top picks are trading near 52-week highs, momentum investing has [proven successful](#) over time, given persistent market trends and behaviors that reinforce the trend. For the new year, consider stocks with robust fundamentals that have proven to deliver results amid macro and geopolitical headwinds. Most of my picks offer tremendous valuations, growth, profitability, and rising earnings revisions. We have many stocks with strong buy recommendations, and you can filter them using [Stock Screens](#) to suit your specific investment objectives. Alternatively, [Alpha Picks](#) might be ideal if you're interested in two monthly stock picks of the top 'strong buy' quant stocks. Seeking Alpha's quant ratings and investment research tools help to ensure you are furnished with the best resources to make informed investment decisions while taking the emotion out of investing. Happy Investing!

Editor's Note: This article discusses one or more securities that do not trade on a major U.S. exchange. Please be aware of the risks associated with these stocks.

I am Steven Cress, Head of Quantitative Strategies at Seeking Alpha. I manage the quant ratings and factor grades on stocks and ETFs in Seeking Alpha Premium. I also lead [Alpha Picks](#), which selects the two most attractive stocks to buy each month and also determines when to sell them.

This article was written by



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Steven Cress is the Head of Quantitative Strategies at Seeking Alpha. He previously founded and ran hedge fund and asset management company Cress Capital Management, and was the Head of International Business Development at Northern Trust. For the majority of his career, he ran a proprietary trading desk at Morgan Stanley.

Steven created and manages the Quant Ratings and Dividend Ratings for stocks, REITs and ETFs in Seeking Alpha Premium. He also leads [Alpha Picks](#), which selects two attractive stocks to buy each month, and also determines when to sell them. [Learn More](#) about Alpha Picks and its performance.

Analyst's Disclosure: I/we have no stock, option or similar derivative position in any of the companies mentioned, and no plans to initiate any such positions within the next 72 hours. I wrote this article myself, and it expresses my own opinions. I am not receiving compensation for it. I have no business relationship with any company whose stock is mentioned in this article.

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Taysha Gene Therapies Reports Fourth Quarter and Full Year 2022 Financial Results and Provides Corporate Update

Mar. 28, 2023 4:01 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Q4: 2023-03-28 Earnings Summary

Transcript

10-K

10-Q

EPS of -\$0.34 misses by \$0.00 | Revenue of \$2.50M misses by \$6.50M

Initiated screening of first potential subject in Phase 1/2 REVEAL trial in Rett syndrome; dosing of first adult patient with TSHA-102 expected in H1 2023; submitted protocol amendment to allow for younger patients; initial available Phase 1/2 clinical data, primarily on safety, expected in H1 2023

Clinical Trial Application (CTA) submission to United Kingdom (UK) MHRA for TSHA-102 in pediatric patients with Rett syndrome expected in mid-2023; Investigational New Drug (IND) application to United States (U.S.) Food and Drug Administration (FDA) in Rett syndrome anticipated in H2 2023

FDA feedback for TSHA-120 in giant axonal neuropathy (GAN) suggests consideration of alternative clinical trial designs for clinically meaningful and objectively measured treatment effects; Company plans to request a formal meeting with FDA to discuss final findings from currently ongoing comprehensive data analyses and potential regulatory path forward in Q2 2023

Conference call and live webcast today at 4:30 PM Eastern Time

DALLAS, March 28, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today reported financial results for the fourth quarter and full-year ended December 31, 2022, and provided a corporate update.

"The actions taken early this year to improve execution and expedite progress on our two lead clinical programs in Rett syndrome and GAN are having a positive effect," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "We recently initiated screening of the first potential adult subject for the REVEAL Rett syndrome trial and remain on track to dose the first patient and deliver initial available first-in-human adult data, primarily on safety, for TSHA-102 in the first half of the year. Additionally, we recently submitted a protocol amendment to allow patients as young as 15 years old to be included in the study, which we believe will further expedite enrollment. We remain on track to submit a CTA to the MHRA in mid-2023 to conduct a pediatric Rett syndrome trial, and plan to submit an IND to the FDA for Rett syndrome in the second half of 2023. For TSHA-120 in GAN, based on the constructive feedback recently received from the FDA in response to our follow up questions to the formal Type B end-of-Phase 2 meeting minutes, coupled with the positive preliminary assessment of the ongoing comprehensive data analyses, we plan to submit a formal meeting request to the Agency in the second quarter of 2023 to discuss the potential regulatory pathway forward for this ultra-rare disease with no approved treatment."

Recent Corporate Highlights

TSHA-102 in Rett syndrome: a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare inherited genetic neurodevelopmental disorder. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular MECP2 expression. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission.

- Screening initiated for first potential adult patient with Rett syndrome for the Phase 1/2 REVEAL trial

- Submitted protocol amendment expanding enrollment eligibility to include subjects ≥ 15 years
- Dosing of the first adult patient with Rett syndrome anticipated in H1 2023
- Initial available Phase 1/2 clinical data, primarily on safety, for TSHA-102 in adult patients with Rett syndrome expected in H1 2023, with planned quarterly updates on available clinical data thereafter
- CTA submission to UK MHRA for TSHA-102 in pediatric patients with Rett syndrome anticipated in mid-2023
- IND application submission to U.S. FDA for Rett syndrome expected in H2 2023
- Continued dosing of adult patients with Rett syndrome in the REVEAL trial in H2 2023

TSHA-120 for giant axonal neuropathy (GAN): a self-complimentary intrathecally delivered AAV9 gene therapy in clinical evaluation for GAN, an ultra-rare inherited genetic neurodegenerative disorder with no approved treatments. TSHA-120 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission.

- Completed CMC module 3 amendment submission to FDA detailing commercial process product manufacturing and drug comparability analysis
- Receipt of FDA's response to Taysha's follow up questions to the formal Type B end-of-Phase 2 meeting minutes
- FDA clarified MFM32, the primary efficacy scale discussed at the FDA Type B end-of-Phase 2 meeting, as a relevant primary endpoint only in the setting of a randomized double blind placebo controlled trial and acknowledged Taysha's challenge in executing and enrolling such a study design due to the ultra-rare nature of GAN
- FDA is open to regulatory flexibility in a controlled trial setting and willing to consider alternative study designs utilizing objective measurements to demonstrate a relatively large treatment effect that is self-evident and clinically meaningful

- Ongoing comprehensive analyses of functional, biological and electrophysiological assessments as part of totality of data evaluation to inform future interactions with the FDA
- Submission of a formal meeting request to the FDA planned in Q2 2023

Fourth Quarter and Full-Year 2022 Financial Highlights

Research and Development Expenses: Research and development expenses were \$13.9 million for the three months ended December 31, 2022, compared to \$37.9 million for the three months ended December 31, 2021. Research and development expenses were \$91.2 million for the full year ended December 31, 2022, compared to \$131.9 million for the full year ended December 31, 2021. The \$40.7 million decrease was primarily attributable to a decrease of \$20.3 million in research and development manufacturing and other raw material purchases and a \$9.0 million decrease in license fees. The decrease in research and development expenses for the year ended December 31, 2022 was also attributable to a \$12.0 million decrease in third-party research and development fees, mainly related to non-clinical studies and toxicology studies and a \$4.7 million decrease in compensation expense as a result of lower headcount. Overall, lower research and development expenses for the year ended December 31, 2022 were partially offset by higher clinical trial expenses of \$2.4 million and higher severance expense of \$2.9 million in 2022.

General and Administrative Expenses: General and administrative expenses were \$7.3 million for the three months ended December 31, 2022, compared to \$11.8 million for the three months ended December 31, 2021. General and administrative expenses were \$37.4 million for the year ended December 31, 2022, compared to \$41.3 million for the year ended December 31, 2021. The decrease of approximately \$3.9 million was primarily attributable to \$5.0 million of lower consulting professional fees and lower compensation expenses driven by lower headcount in 2022. Lower general and administrative expenses were partially offset by \$1.1 million of severance expense.

Net loss: Net loss for the three months ended December 31, 2022 was \$55.7 million, or \$0.99 per share, as compared to a net loss of \$50.4 million, or \$1.32 per share, for the three months ended December 31, 2021. In November 2022, we recorded a \$36.4 million non-cash, non-recurring impairment charge related to the North Carolina manufacturing facility. The net loss for the three months ended December 31, 2022 was partially offset by revenue of \$2.5 million recognized related to the Astellas Transactions. Net loss for the full year ended December 31, 2022 was \$166.0 million or \$3.78 per share, as compared to a net loss of \$174.5 million, or \$4.64 per share, for the full year ended December 31, 2021.

Cash and cash equivalents: As of December 31, 2022, Taysha had \$87.9 million in cash and cash equivalents. The Company continues to expect that its current cash resources will support planned operating expenses and capital requirements into the first quarter of 2024.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 4:30 pm ET to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13736479. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of TSHA-102 and TSHA-120 to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, the potential market opportunity for these product candidates, our corporate growth plans, the forecast of our cash runway. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2022, which is available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

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

	For the three months ended		For the twelve months ended	
	December 31 2022	December 31 2021	December 31 2022	December 31 2021
Revenue:				
Service Revenue	\$ 2,502	-	\$ 2,502	\$ -
Operating expenses:				
Research and development	13,861	37,918	91,169	131,943
General and administrative	7,341	11,806	37,360	41,324
Impairment of long-lived assets	36,420	-	36,420	-
Total operating expenses	57,622	49,724	164,949	173,267
Loss from operations	(55,120)	(49,724)	(162,447)	(173,267)
Other income (expense):				
Interest Income	199	29	249	172
Interest expense	(796)	(691)	(3,798)	(1,428)
Other	(6)	-	(18)	-
Total other income (expense)	(603)	(662)	(3,567)	(1,256)
Net loss	\$ (55,723)	\$ (50,386)	\$ (166,014)	\$ (174,523)
Net loss per common share, basic and diluted	\$ (0.99)	\$ (1.32)	\$ (3.78)	\$ (4.64)
Weighted average common shares outstanding, basic and diluted	56,386,130	38,110,597	43,952,015	37,650,566

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

	December 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 87,880	\$ 149,103
Prepaid expenses and other current assets	8,537	10,499
Total current assets	96,417	159,602
Restricted cash	2,637	2,637
Property, plant and equipment, net	14,963	50,610
Operating lease right-of-use assets	10,943	-
Other noncurrent assets	1,316	1,107
Total assets	\$ 126,276	\$ 213,956
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 10,946	\$ 21,763
Accrued expenses and other current liabilities	18,287	29,983
Deferred revenue	33,557	-
Total current liabilities	62,790	51,746
Build-to-suit lease liability	-	25,900
Term loan, net	37,967	37,192

Operating lease liability, net of current portion	20,440	-
Other noncurrent liabilities	4,130	3,735
Total liabilities	125,327	118,573
<hr/>		
Stockholders' equity		
Common stock, \$0.00001 par value per share; 200,000,000 shares authorized and 63,207,507 issued and outstanding as of December 31, 2022 and 38,473,945 outstanding as of December 31, 2021	1	-
Additional paid-in capital	402,389	331,032
Accumulated deficit	(401,441)	(235,649)
Total stockholders' equity	949	95,383
Total liabilities and stockholders' equity	\$ 126,276	\$ 213,956

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Presentation on New Preclinical Data for TSHA-102 in Rett Syndrome at Upcoming American Society of Gene and Cell Therapy 26th Annual Meeting

Apr. 27, 2023 4:52 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, April 27, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today announced that an abstract related to its TSHA-102 program in Rett syndrome was accepted for presentation at the upcoming American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting, taking place in Los Angeles, CA from May 16-20, 2023. The abstract includes new preclinical data from a Taysha-sponsored study for TSHA-102, a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene.

Details for the presentation are as follows:

Abstract Title: A Human-Ready Regulated AAV9/miniMECP2-miRARE Gene Therapy (TSHA-102) Improves Survival, Weight, and Behavior After Intracerebroventricular (ICV) Dosing in the Neonatal Knockout Rett (RTT) Mouse Model

Presenter: Sarah Sinnett, Ph.D., University of Texas Southwestern Medical Center, Co-Inventor of miRARE technology

Poster Session Date/Time: Friday, May 19 at 12-2 PM PT

Poster Session: Friday Poster Session

Poster Number: 1365

Additional details can be found at the ASGCT 26th Annual Meeting [website](#).

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies to Release First Quarter 2023 Financial Results and Host Conference Call and Webcast on May 11

May 04, 2023 8:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

DALLAS, May 04, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today announced that it will report its financial results for the first quarter ended March 31, 2023, and host a corporate update conference call and webcast on Thursday, May 11, 2023, at 4:30 PM Eastern Time.

Conference Call Details

Thursday, May 11, at 4:30 PM Eastern Time / 3:30 PM Central Time

Toll Free: 855-327-6837

International: 631-891-4304

Conference ID: 10021767

<https://ir.tayshagtx.com/news-events/events-presentations>

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Reports First Quarter 2023 Financial Results and Provides Corporate Update

May 11, 2023 4:01 PM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Q1: 2023-05-11 Earnings Summary

Transcript

10-Q

EPS of -\$0.28 beats by \$0.10 | Revenue of \$4.71M beats by \$3.58M

Screening completed and dosing scheduled for first potential subject in the Phase 1/2 REVEAL trial in Rett syndrome; dosing of first adult patient with TSHA-102 expected in Q2 2023; initial available Phase 1/2 clinical data, primarily on safety, expected in Q2 2023

Clinical Trial Application (CTA) submission to United Kingdom (UK) MHRA for TSHA-102 in pediatric patients with Rett syndrome expected in mid-2023; Investigational New Drug (IND) application to United States (U.S.) Food and Drug Administration (FDA) in Rett syndrome anticipated in H2 2023

New preclinical data for TSHA-102 in Rett syndrome to be presented during a poster presentation at the upcoming American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting

R&D Day in June 2023 will overview new findings from totality of data evaluation, including comprehensive analyses of functional, biological, and electrophysiological assessments of TSHA-120 in giant axonal neuropathy (GAN), and provide an update on TSHA-102 in Rett syndrome

Formal FDA meeting request submission to discuss regulatory path forward for TSHA-120 in GAN expected in Q2 2023; formal meeting anticipated in Q3 2023

Conference call and live webcast today at 4:30 PM Eastern Time

DALLAS, May 11, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today reported financial results for the first quarter ended March 31, 2023, and provided a corporate update.

"We continue to make significant progress with our two lead clinical programs and remain on track to deliver on multiple key milestones, including the generation of first-in-human clinical data for TSHA-102 in adult patients with Rett syndrome, the submission of a CTA to the MHRA to initiate expansion of TSHA-102 in pediatric patients, the submission of an IND application to the FDA for TSHA-102, and obtaining further clarity from the FDA on the regulatory path forward for TSHA-120 in GAN," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "Screening is completed, and dosing is now scheduled for our first potential patient in the adult Rett syndrome study. For GAN, our comprehensive analyses of the totality of data for TSHA-120 continues to be encouraging and includes compelling findings with potential to further support a regulatory path forward."

Sukumar Nagendran, M.D., President, and Head of R&D added, "We plan to seek a formal meeting with the FDA to discuss the totality of findings from the functional, biological, and electrophysiological assessments of TSHA-120 in GAN, anticipated in the third quarter of this year. In the near term, we look forward to hosting an R&D Day in June where we will overview the GAN disease state and share the comprehensive analyses, as well as provide an update on our Rett program. For TSHA-102, new preclinical data supporting the efficacy and safety of TSHA-102 and the miRARE technology in Rett syndrome will be presented as part of a poster presentation at the upcoming ASGCT conference. We believe that the clinical and preclinical data generated to date across our Rett syndrome and GAN programs reinforce our gene therapy approach, and the therapeutic potential to address severe unmet needs in monogenic central nervous system disease."

Recent Corporate Highlights

TSHA-102 in Rett syndrome: a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission.

- Phase 1/2 REVEAL trial in adult patients with Rett syndrome
 - Completed screening and scheduled dosing for first potential adult patient with dosing anticipated in Q2 2023
 - Initial available Phase 1/2 clinical data, primarily on safety, expected in Q2 2023, with planned quarterly updates on available clinical data thereafter
 - Continued dosing of adult patients with Rett syndrome in the REVEAL trial in H2 2023
- CTA submission to UK MHRA for TSHA-102 in pediatric patients with Rett syndrome anticipated in mid-2023
- IND application submission to U.S. FDA for Rett syndrome expected in H2 2023
- New preclinical data for TSHA-102 in Rett syndrome to be presented as a poster presentation at the upcoming ASGCT 26th Annual Meeting on Friday, May 19 at 12-2 PM PT; these data and available clinical data from Phase 1/2 REVEAL trial will be presented in upcoming R&D Day in June

TSHA-120 for giant axonal neuropathy (GAN): a self-complementary intrathecally delivered AAV9 gene therapy in clinical evaluation for GAN, an ultra-rare inherited genetic neurodegenerative disorder with no approved treatments. TSHA-120 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission.

- Completed CMC module 3 amendment submission detailing commercial process product manufacturing and drug comparability analysis; awaiting FDA feedback

- R&D Day in June 2023 to overview new findings from totality of data evaluation, including comprehensive analyses of functional, biological, and electrophysiological assessments of TSHA-120 in GAN
- Submission of a formal meeting request to the FDA in Q2 2023 to discuss alternative study designs, additional objective measures and regulatory path forward; formal meeting anticipated in Q3 2023

First Quarter 2023 Financial Highlights

Research and Development Expenses: Research and development expenses were \$12.5 million for the three months ended March 31, 2023, compared to \$38.2 million for the three months ending March 31, 2022. The \$25.7 million decrease was due to reduced research and development compensation as a result of lower headcount of \$10.7 million. The decrease was also due to reduced manufacturing and other raw material purchases of \$7.1 million. We also incurred \$6.4 million reduced expense in non-clinical studies related to translational and toxicology studies and \$1.5 million lower expense in other research and development activities.

General and Administrative Expenses: General and administrative expenses were \$8.8 million for the three months ended March 31, 2023, compared to \$11.5 million for the three months ended March 31, 2022. The decrease of \$2.7 million was due to reduced general and administrative compensation as a result of lower headcount and reduced consulting and professional fees.

Net loss: Net loss for the three months ended March 31, 2023 was \$17.6 million or \$0.28 per share, as compared to a net loss of \$50.3 million, or \$1.32 per share, for the three months ended March 31, 2022. The net loss for the three months ended March 31, 2023 was partially offset by revenue of \$4.7 million recognized related to the Astellas Transactions.

Cash and cash equivalents: As of March 31, 2023, Taysha had \$63.4 million in cash and cash equivalents. Taysha continues to expect that its current cash resources will support planned operating expenses and capital requirements into the first quarter of 2024.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 4:30 pm ET to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 855-327-6837 (U.S./Canada) or 631-891-4304 (international). The conference ID for all callers is 10021767. The live webcast and replay may be accessed by visiting Taysha's website at <https://ir.tayshagtx.com/news-events/events-presentations>. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

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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, 2023	For the three months ended March 31, 2022
Revenue:		
Service Revenue	\$ 4,706	\$ -
Operating expenses:		
Research and development	12,514	38,182
General and administrative	8,751	11,469
Total operating expenses	21,265	49,651
Loss from operations	(16,559)	(49,651)
Other income (expense):		
Interest Income	319	14
Interest expense	(1,374)	(672)
Other expense	(8)	(8)
Total other expense	(1,063)	(666)
Net loss	\$ (17,622)	\$ (50,317)
Net loss per common share, basic and diluted	\$ (0.28)	\$ (1.32)
Weighted average common shares outstanding, basic and diluted	63,260,905	38,174,717

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

	December March 31, 2023	31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 63,425	\$ 87,880
Prepaid expenses and other current liabilities	8,933	8,537
Total current assets	72,358	96,417
Restricted cash	2,637	2,637
Property, plant and equipment, net	14,642	14,963
Operating lease right-of-use assets	10,647	10,943
Other noncurrent assets	1,316	1,316
Total assets	\$ 101,600	\$ 126,276
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	\$ 9,002	\$ 10,946
Accrued expenses and other current liabilities	16,602	18,287
Deferred revenue	28,851	33,557
Total current liabilities	54,455	62,790
Term loan, net	38,161	37,967

Operating lease liability, net of current portion	19,928	20,440
Other noncurrent liabilities	4,004	4,130
Total liabilities	116,548	125,327

Preferred stock, \$0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of March 31, 2023 and December 31, 2022

Stockholders' (deficit) equity

Common stock, \$0.00001 par value per share; 200,000,000 shares authorized and 63,473,349 and 63,207,507 issued and outstanding as of March 31, 2023 and December 31, 2022, respectively

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Additional paid-in capital	404,114	402,389
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Accumulated deficit	(419,063)	(401,441)
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Total stockholders' (deficit) equity	(14,948)	949
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Total liabilities, convertible preferred stock, and stockholders' (deficit) equity

\$ 101,600 \$ 126,276

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Presents Preclinical Data on TSHA-102 for Rett Syndrome Demonstrating Cellular Regulation of MeCP2 Expression in Key Mouse Models at the American Society of Gene and Cell Therapy 26th Annual Meeting

May 19, 2023 8:00 AM ET | Taysha Gene Therapies, Inc. (TSHA)

New preclinical data after neonatal administration in wild-type mice showed no detectable impact on survival, neurobehavioral functions and overall health, suggesting TSHA-102, engineered with novel miRARE technology, avoided toxic overexpression of MeCP2 within cells already expressing MeCP2

Data reinforce previous findings in $Mecp2^{-/-}$ knockout mice demonstrating TSHA-102 regulated cellular MeCP2 levels and significantly improved survival, overall neurobehavioral function and growth

Data in neonatal mouse models highlight the potential of the miRARE technology to enable safe expression levels of MeCP2, which may address the risks associated with both under and overexpression of MeCP2 resulting from the mosaic pattern of MECP2 silencing in females with Rett syndrome

Dosing of the first adult patient with TSHA-102 in the Phase 1/2 REVEAL trial in Rett syndrome is expected in Q2 2023

DALLAS, May 19, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today presents preclinical data from neonatal mouse models on TSHA-102 for Rett syndrome, including new data in wild-type mice, at the American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting. TSHA-102 utilizes a mini*MECP2* gene and a novel miRNA-Responsive Auto-Regulatory Element (miRARE) technology designed to regulate cellular *MECP2* expression. In a Taysha-sponsored study, the safety and efficacy of TSHA-102 were explored in both neonatal wild-type and *Mecp2*^{-/-} knockout mice, respectively. Preclinical in-life data on early intervention of TSHA-102 in neonatal mice suggest miRARE enables the expression of the MeCP2 protein in deficient CNS cells while preventing toxic overexpression within cells expressing normal levels of MeCP2.

"These encouraging new preclinical data in wild-type mice indicate that TSHA-102, engineered with our miRARE technology, avoided overexpression of MeCP2 within cells already expressing MeCP2, while maintaining normal survival, neurobehavioral function and overall health," said Sukumar Nagendran, M.D., President, and Head of R&D. "These new data augment previous findings in the *Mecp2*^{-/-} knockout mouse model, suggesting that TSHA-102 regulated expression of *MECP2* in both normal and *MECP2* deficient cells, which is critical given that Rett syndrome represents such a challenging case for human gene therapy because the therapeutic window for *MECP2* transgene expression is narrow. Either *MECP2* deficiency or duplication can lead to serious neurodevelopmental disease. We believe these new data from neonatal wild-type mice support the potential of miRARE to enable the optimal amount of MeCP2. This would be critical to modulating the cellular expression of MeCP2 in an appropriate, clinically relevant manner, given the mosaic pattern of *MECP2* silencing characteristic of female patients with Rett syndrome."

Sarah Sinnett, Ph.D., University of Texas Southwestern Medical Center, Co-Inventor of miRARE technology, added, "TSHA-102 pairs a therapeutic gene with miRARE, all within a single vector genome. The miRARE technology was designed to mitigate the risk of MeCP2 overexpression through a post-transcriptional feedback repression mechanism. We are pleased that miRARE permitted efficacy in *Mecp2*^{-/-} mice without compromising safety in wild-type mice. Importantly, these findings could translate into clinical benefits for treating patients with Rett syndrome."

Preclinical data in neonatal wild-type mice suggest miRARE suppressed toxic overexpression after early intervention with TSHA-102:

- In wild-type mice treated with TSHA-102, new data showed no deleterious impact on survival, neurobehavioral functions and overall health, suggesting miRARE regulated expression of MeCP2 with the below results from the study:
 - No toxicity relative to vehicle treatment
 - No reduction in survival over 36-weeks
 - No treatment effect on Bird Score (a measure of Rett syndrome-like behaviors and pathologies) analysis relative to vehicle treatment
 - No impact on overall growth over the course of the study

This builds on prior preclinical data in neonatal *Mecp2*^{-/-} knockout mice showing miRARE regulated *MECP2* expression levels in deficient CNS cells with early intervention of TSHA-102:

- In *Mecp2*^{-/-} knockout mice (mouse model recapitulating developmental, physiological, and behavioral features of human Rett syndrome) treated with TSHA-102 with the below results from the study:
 - 47% survived the 36-week study vs a median survival of 8.1 weeks with vehicle-treated knockout mice, representing a significant ($p<0.0001$) >4-fold extension of their lifespan
 - Restoration of normal and faster-than-normal growth
 - Aggregate Bird Score was significantly improved at several time points, with a significant delay in the onset of severe Rett syndrome-like phenotypes, including the delayed average age of onset for severe clasping from approximately 7 to 21 weeks and severely abnormal gait from approximately 8 to 20 weeks

TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy in clinical evaluation for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 is currently being evaluated in the Phase 1/2 REVEAL trial in adult patients with Rett syndrome. The dosing of the first adult patient with TSHA-102 is expected in Q2 2023, with initial available clinical data, primarily on safety, anticipated thereafter in Q2 2023. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) and has been granted Orphan Drug designation from the European Commission for the treatment of Rett syndrome.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Announces First Patient Dosed with TSHA-102 in the REVEAL Phase 1/2 Trial Under Investigation for the Treatment of Rett Syndrome

Jun. 05, 2023 8:00 AM ET | Taysha Gene Therapies, Inc. (TSHA)

The Phase 1/2 REVEAL trial is a first-in-human, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adults with Rett syndrome

TSHA-102 utilizes novel miRARE technology, designed to regulate cellular MECP2 levels

Initial available clinical safety data from Phase 1/2 REVEAL trial will be reported at Taysha's upcoming R&D Day on June 28, 2023, at 10:00 AM Eastern Time

DALLAS, June 05, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. ([TSHA](#)), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today announced that the first patient has been dosed 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. TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy that utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. The study is being conducted at CHU Sainte-Justine, the Université de Montréal mother and child university hospital centre in Montreal, Canada.

"Dosing of the first adult patient marks the beginning of clinical evaluation of TSHA-102 in the Phase 1/2 REVEAL trial, and, to our knowledge, the first time a gene therapy has ever been evaluated in a clinical setting for the treatment of Rett syndrome," said Sukumar Nagendran, M.D., President, and Head of R&D. "By targeting the regulation of gene expression on a cell-by-cell basis, we believe our miRARE technology has the ability to enable safe expression of *MECP2*, which may help address the risks associated with both under and overexpression resulting from the mosaic pattern of *MECP2* silencing. This is a significant milestone that furthers our quest to bring a potentially transformational gene therapy to patients and families living with Rett syndrome. We look forward to sharing initial available clinical safety data from the Phase 1/2 REVEAL trial at our R&D Day on June 28, 2023."

The [Phase 1/2 REVEAL trial](#) is a first-in-human, open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adult females with Rett syndrome due to *MECP2* loss-of-function mutation. Participants will receive a single lumbar intrathecal injection of TSHA-102. Dose escalation will evaluate two dose levels of TSHA-102 sequentially, with an initial dose of 5×10^{14} total vector genomes (vg) and the second dose of 1×10^{15} vg. The maximum tolerated dose (MTD) or maximum administered dose (MAD) established will then be administered during dose expansion. Per the protocol, an independent data monitoring committee will review available safety data from the first patient at approximately six weeks post-dosing to determine if the Company can proceed with dosing the second patient. Initial available clinical safety data will be reported at Taysha's upcoming R&D Day on June 28, 2023. To register for the event, please click [here](#).

Elsa Rossignol, M.D., FRCP, FAAP, Associate Professor Neuroscience and Pediatrics, and Principal Investigator of the REVEAL study added, "Based on its unique and compelling technology targeting the genetic root cause of Rett syndrome, TSHA-102 has the potential to transform care by addressing a significant unmet medical need for patients with this devastating and currently incurable disease. The dosing of the first patient in this important clinical trial represents a critical advancement in evaluating the potential of gene therapy for Rett syndrome. It is a privilege to be part of this important endeavor. In the name of all affected families, I thank Taysha for bringing this potentially transformative therapy from the bench to the bedside."

Sabrina Millson, President of Ontario Rett Syndrome Association further added, "This is a momentous day for the Rett syndrome community. As a mom to a daughter living with Rett syndrome and the president of the Ontario Rett Syndrome Association here in Canada, I know first-hand how this disease leads to debilitating symptoms, including difficulties in communication, mobility and breathing. The potential for a treatment that addresses the underlying cause of disease and slows progression or potentially prevents the onset of disease with early intervention is truly remarkable. We're pleased to collaborate with Taysha Gene Therapies in an effort to bring a gene therapy treatment that could meaningfully change the lives of patients and their caregivers."

About TSHA-102

TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy in clinical evaluation for Rett syndrome. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the United States (U.S.) Food and Drug Administration (FDA) and has been granted Orphan Drug designation from the European Commission. We are advancing TSHA-102 in the REVEAL Phase 1/2 clinical trial under a CTA approved by Health Canada. A CTA submission to United Kingdom (UK) MHRA in pediatric patients with Rett syndrome is expected in mid-2023, and an Investigational New Drug (IND) application to the FDA is anticipated in the second half of 2023.

About Rett Syndrome

Rett syndrome is a rare neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene, which is a gene that's essential for neuronal and synaptic function in the brain. The disorder is characterized by intellectual disabilities, loss of communication, seizures, slowing and/or regression of development, motor and respiratory impairment, and shortened life expectancy. Rett syndrome primarily occurs in females and is one of the most common genetic causes of severe intellectual disability. Currently, there are no approved disease-modifying therapies that treat the genetic root cause of the disease. Rett syndrome caused by a pathogenic/likely pathogenic *MECP2* mutation is estimated to affect between 15,000 and 20,000 patients in the U.S., EU and UK.

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About the CHU Sainte-Justine

The Centre hospitalier universitaire Sainte-Justine is the largest mother-child hospital in Canada. A member of the Université de Montréal extended network of excellence in health (RUIS), CHU Sainte-Justine has 6759 employees, including 1770 nurses and nursing assistants; 1131 other healthcare professionals; 531 physicians, dentists and pharmacists; 931 residents and over 280 researchers; 170 volunteers; and 3 406 interns and students in a wide range of disciplines. CHU Sainte-Justine has 484 beds, including 67 at the Centre de réadaptation Marie Enfant (CRME), the only exclusively pediatric rehabilitation centre in Québec. The World Health Organization has recognized CHU Sainte-Justine as a "health-promoting hospital." chusj.org

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies to Host Virtual R&D Day on Lead Clinical Investigational Programs TSHA-120 in Giant Axonal Neuropathy (GAN) and TSHA-102 in Rett Syndrome

Jun. 15, 2023 8:00 AM ET | **Taysha Gene Therapies, Inc. (TSHA)**

Virtual R&D Day featuring collaborator Salman Bhai, MD, and Taysha's leadership team at 10:00 AM ET on June 28, 2023

Company to provide update on new data analyses for TSHA-120 in GAN, and initial safety observations for TSHA-102 in Rett syndrome

DALLAS, June 15, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies ([TSHA](#)), Inc. , a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), today announced it will host a virtual R&D Day on Wednesday, June 28, 2023 at 10:00 AM ET to discuss updates on TSHA-120, a self-complimentary intrathecally delivered investigational AAV9 gene therapy in clinical evaluation for GAN, and TSHA-102, a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy in clinical evaluation for Rett syndrome.

The event will feature collaborator Salman Bhai, MD, Assistant Professor of Neurology at UT Southwestern Medical Center, who will discuss the disease course and biology of GAN and present new data and analyses from the ongoing natural history and interventional trial evaluating TSHA-120. In addition, Taysha leadership will provide a clinical update on the investigational TSHA-102 program, including the initial safety observations of TSHA-102 from the first patient recently dosed in the [Phase 1/2 REVEAL trial](#). The REVEAL trial is evaluating the safety and preliminary efficacy of TSHA-102 in adult females with Rett syndrome. More detailed clinical updates on the first patient will be provided in the third quarter of this year following the initial review of available safety data by the Independent Data Monitoring Committee.

A live question and answer session will follow the formal presentations. To register for the event, please click [here](#).

About Salman Bhai, MD

Dr. Bhai is an Assistant Professor in the Department of Neurology at UT Southwestern Medical Center and the Director of the Neuromuscular Center in the Institute for Exercise and Environmental Medicine at Texas Health Presbyterian Hospital Dallas. He specializes in neuromuscular disorders. Dr. Bhai earned his medical degree at Harvard Medical School. He completed his residency in neurology through Harvard Medical School at Brigham and Women's Hospital and Massachusetts General Hospital, where he also received advanced training through a fellowship in neuromuscular medicine and earned a medical education certificate. He is board certified by the American Board of Psychiatry and Neurology in neurology and neuromuscular medicine as well as by the American Board of Electrodiagnostic Medicine. He joined the UT Southwestern faculty in 2020. He is a member of the American Academy of Neurology, the Dallas County Medical Society, and the Texas Neurological Society. Dr. Bhai's clinical interests include the evaluation and treatment of neuromuscular disorders. He focuses on patients with hereditary and autoimmune neuromuscular disorders. Dr. Bhai's research focuses on understanding metabolic and mitochondrial dysfunction in muscle disorders. He is the principal investigator for multiple clinical trials in neuromuscular diseases. He serves as an organizer and a participant for European Neuromuscular Center expert workshops. He has been an invited lecturer nationally and internationally in his areas of expertise. As a clinician-scientist and educator, Dr. Bhai strives to improve the lives of patients and their families.

About Taysha Gene Therapies

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Provides Clinical Updates for Investigational Programs TSHA-120 in Giant Axonal Neuropathy (GAN) and TSHA-102 in Rett Syndrome at R&D Day

Jun. 28, 2023 8:00 AM ET | Taysha Gene Therapies, Inc. (TSHA)

Company views that results of comprehensive data analysis of TSHA-120 and development of disease progression model (DPM) address U.S. Food and Drug Administration (FDA) feedback regarding the effort-dependent nature of MFM32 as primary endpoint in an unblinded study and heterogeneity of GAN; Taysha plans to review potential regulatory pathway for TSHA-120 at a formal meeting with the FDA expected in Q3 2023

New GAN analysis identified multiple functional, electrophysiological and biological measurements that demonstrate a clinically meaningful and objective measurement of TSHA-120 treatment effect on disease progression

Encouraging initial clinical observations seen in the first adult patient with Rett syndrome recently dosed with TSHA-102 in REVEAL Phase 1/2 trial; safety and efficacy update and Independent Data Monitoring Committee (IDMC) approval to dose second patient expected in early Q3 2023

Detailed updates will be presented at virtual R&D Day today at 10:00 AM ET

DALLAS, June 28, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), announced new data analyses for TSHA-120 in GAN and initial clinical observations for TSHA-102 in Rett syndrome. Taysha will host a virtual R&D Day today at 10:00 AM ET to discuss these updates. The webcast link can be accessed on the [Events and Presentations](#) section of Taysha's website.

"Late last year, the company submitted and discussed with the FDA a subset of available evidence supporting the potential therapeutic benefit and safety profile for TSHA-120 in patients with GAN, an ultra-rare disease with currently no approved treatments. FDA feedback included the need to address the heterogeneity of disease progression in GAN and the effort-dependent nature of MFM32 as a primary endpoint, considering the unblinded study design," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "Given the FDA also indicated it is open to regulatory flexibility in a controlled trial setting and willing to consider alternative study designs, we undertook an extensive analysis of the totality of data available to determine a feasible regulatory path forward for TSHA-120."

Mr. Nolan continued, "We believe the new analyses may help support an approval pathway for TSHA-120 for the treatment of GAN. Our newly developed disease progression model demonstrates predictable and homogenous disease progression in classic GAN, which in our view supports the use of natural history data as an external control. Additionally, we identified objective functional, electrophysiological and biological measurements that demonstrated a clinically meaningful treatment effect, which is also accompanied by over seven years of clinical data supporting the safety profile. We've requested a formal FDA meeting to discuss these new developments to support a potential regulatory path forward for TSHA-120. We expect the meeting to take place in the third quarter of this year."

"For our TSHA-102 program in Rett syndrome, we are encouraged by the initial clinical observations of the first adult patient recently dosed in the REVEAL Phase 1/2 trial," said Sukumar Nagendran, M.D., President, and Head of R&D. "We look forward to providing further clinical updates on the safety and efficacy observations for the first patient early in the third quarter of this year, following the required IDMC adjudication of the initial clinical data. Subsequent REVEAL trial updates will be provided quarterly, thereafter. We remain on track to submit a CTA to the UK MHRA in pediatric patients in mid-2023 and to submit an IND application to the FDA in the second half of 2023."

Key R&D Day Highlights

TSHA-120: a self-complimentary intrathecally delivered AAV9 gene therapy being evaluated in an open-label, dose-escalation, non-randomized Phase 1/2 trial for GAN, an ultra-rare inherited genetic neurodegenerative disorder with no approved treatments.

- New comprehensive data analysis enabled the development of a DPM using all available data from the largest existing GAN natural history database; DPM demonstrates a predictable and homogenous disease progression in classic GAN, which supports the potential for natural history data to serve as a suitable external control
- Given patient age and the extensive and wide-spread damage to the central nervous system as well as a length-dependent progression in the peripheral nervous system, a more positive treatment impact is expected in outcomes related to the arms compared to the legs; the longer the disease progresses, the greater the degeneration with decreasing likelihood of impacting the disease
- Relatively stable to improved sensory response amplitudes observed on nerve conduction studies, in conjunction with increased regenerative clusters on nerve biopsy, suggest sensory nerve or neuron regeneration in a progressive neurodegenerative disease
- Using natural history data as an external control, Bayesian analysis demonstrated a clinically meaningful treatment effect of TSHA-120 as measured through the slowing of disease progression observed across multiple functional, electrophysiological and biological measures:
 - Functional endpoints:

- Modified Friedreich's Ataxia Rating Scale (mFARS) demonstrated a 99% probability of positive treatment effect on slowing disease progression, with an estimated average treatment effect of 31%
- Motor Function Measure 32 (MFM32) Domain 3 (distal motor function – hands) demonstrated a 99% probability of positive treatment effect on slowing disease progression, with an estimated treatment effect of 28%
- Visual Acuity, as measured by Logarithm of the Minimum Angle of Resolution (LogMAR), demonstrated 100% probability of positive treatment effect on slowing disease progression, with an estimated treatment effect of 70% in the right eye and 51% in the left eye
- Electrophysiological endpoints:
 - Analysis demonstrated a 100% probability of positive treatment effect on slowing disease progression, with an estimated treatment effect of 189% and 152% for Ulnar Sensory Nerve Action Potential (SNAP) and median SNAP amplitude, respectively, indicating disease improvement
 - Compound Muscle Action Potential (CMAP) demonstrated a 94% probability of positive treatment effect on slowing disease progression, with an estimated 29% treatment effect
- Biological Endpoints:
 - 4 out of the 5 patients that had stabilization or improvements in SNAPS had increased regenerative clusters on nerve biopsy
 - Skin biopsy-nerve fiber density: 5 patients saw stabilization or increases in nerve fiber density of the skin in at least one location of the proximal or distal leg at month 12, including 3/3 in the high-dose and one in the medium-high dose
- Over seven years of long-term clinical data support the safety and tolerability profile of TSHA-120
- New data analysis will help inform discussion with the FDA regarding a regulatory path forward for TSHA-120; formal meeting with FDA expected in the third quarter of 2023

TSHA-102: a self-complementary intrathecally delivered AAV9 gene transfer therapy being evaluated in the first-in-human, open labeled, randomized dose escalation and expansion REVEAL Phase 1/2 trial for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression.

- First patient has been dosed in the REVEAL Phase 1/2 trial in adult patients with Rett syndrome being conducted at CHU Sainte-Justine, the Université de Montréal mother and child university hospital centre in Montreal, Canada
 - The patient was discharged from the hospital and has completed multiple follow-up visits, per the study protocol. Additional safety and efficacy updates on the first patient are expected in the early third quarter of 2023, following initial review of available safety data by the IDMC
 - Second potential patient has been identified and will undergo screening if all protocol defined criteria are met; dosing expected to proceed pending IDMC review of available clinical data from the first patient
- CTA submission to UK MHRA in pediatric patients anticipated in mid-2023
- IND application submission to U.S. FDA expected in the second half of 2023

About Taysha Gene Therapies

Taysha Gene Therapies (Nasdaq: TSHA) is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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Source: Taysha Gene Therapies, Inc. 2023 GlobeNewswire, Inc.

Taysha Gene Therapies Announces Positive Recommendation from Independent Data Monitoring Committee of REVEAL Phase 1/2 Trial in Rett Syndrome

Jul. 31, 2023 8:00 AM ET | Taysha Gene Therapies, Inc. (TSHA)

Independent Data Monitoring Committee recommended REVEAL Phase 1/2 trial continuation and proceeding with dosing of second patient based on encouraging initial clinical data from the first adult with Rett syndrome dosed with investigational gene therapy TSHA-102

Initial clinical update from the first patient dosed with TSHA-102 planned for forthcoming quarterly earnings call

Dosing of second patient expected in the third quarter of 2023

DALLAS, July 31, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies ([TSHA](#)), Inc. , a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system (CNS), announced today that the Independent Data Monitoring Committee (IDMC) recommended the continuation of the REVEAL Phase 1/2 trial and that dosing of the second patient in the first cohort can proceed. The decision follows a pre-specified IDMC review of initial clinical data from the first patient dosed with TSHA-102 following the 42-day evaluation period.

"We thank the IDMC members for their guidance and are pleased with their recommendation to continue the REVEAL Phase 1/2 trial," said Sukumar Nagendran, M.D., President and Head of R&D of Taysha. "This recommendation was based on the analysis of initial clinical data from the first adult patient with Rett syndrome to receive TSHA-102. A second patient is expected to be dosed in the third quarter of this year. We are highly encouraged by the initial clinical observations, which support the transformative potential of TSHA-102 and mark important progress in our efforts to bring a gene therapy to patients and families living with Rett syndrome. We look forward to providing an initial clinical update on the first patient at our second quarter corporate update conference call in mid-August."

The [REVEAL Phase 1/2 trial](#) is a first-in-human, open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adult females with Rett syndrome due to *MECP2* loss-of-function mutation. TSHA-102 is administered as a single lumbar intrathecal injection. Dose escalation will evaluate two dose levels of TSHA-102 sequentially. The maximum tolerated dose (MTD) or maximum administered dose (MAD) established will then be administered during dose expansion.

About TSHA-102

TSHA-102 is an investigational self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission. TSHA-102 is being evaluated in the first-in-human, open label, randomized, dose escalation and dose-expansion REVEAL Phase 1/2 trial for adult female patients with Rett syndrome.

About Rett Syndrome

Rett syndrome is a rare neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene, which is a gene that's essential for neuronal and synaptic function in the brain. The disorder is characterized by intellectual disabilities, loss of communication, seizures, slowing and/or regression of development, motor and respiratory impairment, and shortened life expectancy. Rett syndrome primarily occurs in females and is one of the most common genetic causes of severe intellectual disability. Currently, there are no approved disease-modifying therapies that treat the genetic root cause of the disease. Rett syndrome caused by a pathogenic/likely pathogenic *MECP2* mutation is estimated to affect between 15,000 and 20,000 patients in the U.S., EU and UK.

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