Astellas and Taysha Gene Therapies Announce Strategic Investment to Support Development of Taysha's AAV-based Gene Therapy Programs

- Taysha Gene Therapies 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. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") and Taysha Gene Therapies, Inc. (NASDAQ: TSHA, CEO: RA Session II, "Taysha") 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 goa of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements (Taysha)
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 in GAN and TSHA-102 in Rett syndrome, to positively impact quality of life and alter the course of disease in the patients we seek to treat, the potential benefits of Taysha's collaboration with Astellas, the potential for Astellas to exercise any of the options granted to it by Taysha, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, and the 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, 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.

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.

About Astellas Gene Therapies
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
In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties.

Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.

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