Press Release

# Astellas and Kate Therapeutics Announce Exclusive License Agreement for KT430

*KT430 is a preclinical, next-generation investigational gene therapy to treat X-linked myotubular myopathy (XLMTM)*

**TOKYO and SAN DIEGO, June 8, 2023** – Astellas Pharma Inc. (TSE: 4503, President and CEO: Naoki Okamura, "Astellas"), and Kate Therapeutics ("KateTx") today announced an exclusive license agreement to develop and commercialize KT430. KT430 is a preclinical next-generation investigational gene therapy that delivers a functional copy of the *MTM1* gene via a novel MyoAAV capsid to treat X-linked myotubular myopathy (XLMTM), a serious, life-threatening, rare neuromuscular disease characterized by extreme muscle weakness, respiratory failure and early death.

Under the terms of the agreement, Astellas will make an undisclosed upfront payment to KateTx, which is also eligible to receive development, regulatory and commercial milestone payments, plus royalties on worldwide sales. Astellas will receive an exclusive worldwide license to develop, manufacture and commercialize KT430.

"This agreement brings together Astellas and KateTx's collective patient-focused missions, allowing us to evaluate how to advance this new potential therapy for people diagnosed with XLMTM," stated Adam Pearson, Chief Strategy Officer at Astellas. "The combination of Kate's unique scientific approach and Astellas' depth of experience in developing gene therapies for XLMTM provides a solid foundation for the advancement of KT430 as it progresses towards the clinic. The addition of this new potential gene therapy treatment for XLMTM along with our current AT132 program further enhances our commitment to this patient community and dedication to delivering transformative medicines."

"We are very enthusiastic that Astellas has acquired worldwide rights to KT430 and what it means for the patients and families waiting for a potential new therapy for XLMTM," said Kevin Forrest, Ph.D., president and CEO of KateTx. "Our company uses novel technology platforms that directly address the key limitations of current gene therapies, including tissue-specific delivery and gene regulation, with the potential to improve efficacy and safety. We see tremendous opportunities to bring important new therapies to patients with very serious conditions like XLMTM, as well as for other genetic disorders that are the focus of our internal efforts."

**About X-linked Myotubular Myopathy**
XLMTM is a serious, life-threatening, rare neuromuscular disease that is characterized by extreme muscle weakness, respiratory failure and early death. Mortality rates are estimated to be 50 percent in the first 18 months of life. For those patients who survive past infancy, there is an estimated additional 25 percent mortality by the age of 10. XLMTM is caused by mutations in the *MTM1* gene that lead to a lack or dysfunction of myotubularin, a protein that is needed for normal development, maturation and function of skeletal muscle cells. The disease affects approximately 1 in 40,000 to 50,000 newborn males.

XLMTM places a substantial burden of care on patients, families and the healthcare system, including high rates of healthcare utilization, hospitalization and surgical intervention. More than 80 percent of XLMTM patients require ventilator support, and the majority of patients require a gastrostomy tube for nutritional support. In most patients, normal developmental motor milestones are delayed or never achieved. Currently, only supportive treatment options, such as ventilator use or a feeding tube, are available.

**About Kate Therapeutics**
Kate Therapeutics (KateTx) is a patient-focused biotechnology company developing adeno-associated virus (AAV)-based gene therapies to treat genetically defined muscle and heart diseases. The Company is applying novel technology platforms that directly address the key limitations of current gene therapies, including tissue-specific delivery and gene regulation. These breakthroughs have the potential to improve the efficacy and safety of gene therapies and enable the pursuit of a broader set of targets that are otherwise difficult to drug with current technologies. For more information, please visit KateTx's website at https://www.katetherapeutics.com/.

**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., Sanford, N.C. and Tsukuba, Japan.

**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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**Contacts for inquiries or additional information:**
Astellas Pharma Inc.

Portfolio Communications
Sara Zelkovic
+1-347-226-1459
Sara.zelkovic@astellas.com

Corporate Communications
+81-3-3244-3201

**Kate Therapeutics**
Kathy Vincent
Greig Communications Inc.
kathy@greigcommunications.com