Press Release

Astellas Announces Hold Lifted by FDA on FORTIS Clinical Trial of AT845 Investigational Treatment for Adult Patients with Late-Onset Pompe Disease

TOKYO, January 20, 2023 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold on January 19th for the FORTIS Ph1/2 clinical trial evaluating the safety, tolerability and efficacy of investigational AT845 in adults with late-onset Pompe disease (LOPD).

"With that same spirit and focus on patient safety, we look forward to resuming the FORTIS clinical trial and the continued development of AT845 as an important potential new treatment for adults living with LOPD," said Ha Tran, Executive Medical Director for Astellas. "As always, we are grateful to the patients participating in the FORTIS clinical trial and we remain committed to developing novel therapies for those with a high unmet medical need."

Following the clinical hold lift, Astellas is working on completing the clinical and regulatory activities necessary to resume dosing in the FORTIS clinical trial.

The clinical hold lift will have no impact on Astellas' financial forecasts of the current fiscal year ending March 31, 2023**.**

For more information, please see the press release "Astellas Announces FDA Update on the FORTIS Clinical Trial of AT845 in Adults with Late-Onset Pompe Disease" issued on June 27, 2022.

About Pompe Disease
Pompe disease is a rare, severe, autosomal recessive metabolic disease characterized by progressive muscular degeneration. The overall incidence is estimated to be approximately 1 in 40,000 births[1], although frequency and disease progression varies with age of onset, ethnicity and geography[2]. The disease is caused by mutations in the *acid alpha-glucosidase (GAA)* gene that prevent the production and function of a protein called acid alpha-glucosidase (GAA). GAA is responsible for metabolizing glycogen, and dysfunction or absence of this protein results in the accumulation of glycogen in tissues, primarily in the skeletal and cardiac muscles, where it causes damage to tissue structure and function. Currently, the only approved treatment for Pompe is enzyme replacement therapy (ERT), which is a chronic treatment delivered in bi-weekly infusions and relies solely on tissue uptake of GAA from plasma.

About AT845 for the treatment of Late-Onset Pompe Disease (LOPD)
Astellas is developing AT845, a novel gene replacement therapy using an AAV8 vector under a muscle-specific promotor to deliver a functional copy of the *GAA* gene, for the treatment of adult LOPD. AT845 is being investigated to determine whether it can deliver a functional *GAA* gene that is efficiently transduced to express GAA directly in tissues affected by the disease, including skeletal and cardiac muscle.

About FORTIS
FORTIS (NCT04174105) is a multicenter, open-label, ascending dose Phase I/II first-in-human clinical trial to determine if AT845 is safe and tolerable in adults with Late-Onset Pompe Disease (LOPD). The primary endpoints

of the trial are safety and tolerability, as well as efficacy measures, including change in muscle GAA protein expression and enzyme activity from baseline. Secondary endpoints evaluate improvements in respiratory, endurance and quality of life measures.

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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