Press Release

Astellas Announces Topline Results from Long-Term Phase 3 Safety Study of Fezolinetant which Inform Future Regulatory Filings for the Treatment of Vasomotor Symptoms Associated with Menopause

TOKYO, March 7, 2022 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") today announced topline results from the Phase 3 SKYLIGHT 4™ clinical trial investigating the long-term safety of fezolinetant, an investigational oral, nonhormonal compound being studied for the treatment of moderate to severe vasomotor symptoms associated with menopause (VMS) which will support future regulatory filing submissions. VMS, characterized by hot flashes (also called hot flushes) and/or night sweats, are common symptoms of menopause.[1,2]

SKYLIGHT 4 is a randomized, placebo-controlled, double-blind Phase 3 clinical trial in over 1,800 women investigating the long-term (52-week) safety of fezolinetant in women seeking treatment for relief of VMS associated with menopause. The study's primary objectives were to evaluate the effect of fezolinetant on endometrial health and the long-term safety and tolerability of fezolinetant. The primary endpoint assessing endometrial health was achieved and the most common treatment emergent adverse events (TEAE) were headache and COVID-19, consistent with placebo. The topline data further characterize the long-term safety profile of fezolinetant and will inform future regulatory filings. Detailed results will be submitted for publication and for consideration at upcoming medical meetings.

"Based on our initial assessment, we are pleased with the outcome of the SKYLIGHT 4 study, which further characterizes the long-term safety of fezolinetant," said Nancy Martin, M.D., PharmD, Vice President, Global Medical Head, Medical Specialties, Astellas. "With these fezolinetant data, we are hopeful that we will have the opportunity to deliver a first-in-class, nonhormonal treatment option for moderate to severe VMS associated with menopause."

"Vasomotor symptoms are often reported as the most bothersome symptoms of menopause, yet there has been very little innovation in this therapeutic area," said Genevieve Neal-Perry, M.D., Ph.D., Chair, UNC School of Medicine Department of Obstetrics and Gynecology. "I am excited by the potential of a new nonhormonal treatment option for women experiencing moderate to severe VMS associated with menopause."

The SKYLIGHT 4 findings, along with the results from two pivotal Phase 3 clinical trials, SKYLIGHT 1™ and SKYLIGHT 2™, will provide the foundational data for regulatory submissions in the U.S. and Europe.

Fezolinetant is an investigational selective neurokinin-3 (NK3) receptor antagonist. The safety and efficacy of fezolinetant are under investigation and have not been established. If approved by regulatory authorities, fezolinetant would be a first-in-class, nonhormonal treatment option to reduce the frequency and severity of VMS associated with menopause.

About BRIGHT SKY™ Phase 3 Program
The BRIGHT SKY pivotal trials, SKYLIGHT 1™ (NCT04003155) and SKYLIGHT 2™ (NCT04003142), enrolled over 1,020 women with moderate to severe VMS. The trials are double-blinded and placebo-controlled for the first 12 weeks followed by 40-week active treatment extension period. Women were enrolled at over 280 sites within the U.S., Canada and Europe. SKYLIGHT 4™ (NCT04003389) is a 52-week double-blinded and placebo-controlled study designed to investigate long-term safety of fezolinetant. For SKYLIGHT 4, over 1,800 women with VMS were enrolled at over 180 sites within the U.S., Canada and Europe.

About VMS Associated with Menopause
VMS, characterized by hot flashes (also called hot flushes) and/or night sweats are common symptoms of menopause.[1,2] Worldwide, more than half of women 40 to 64 years of age experience VMS and, in the U.S., about 60% to 80% of women experience these symptoms during or after the menopausal transition.[3,4,5,6] VMS can have a disruptive impact on women's daily activities and overall quality of life.[1]

About Fezolinetant
Fezolinetant is an investigational, oral nonhormonal therapy in clinical development for the treatment of moderate to severe VMS associated with menopause. Fezolinetant works by blocking neurokinin B (NKB) binding on the kisspeptin/neurokinin/dynorphin (KNDy) neuron to moderate neuronal activity in the thermoregulatory center of the brain (the hypothalamus) to reduce the frequency and severity of moderate to severe VMS associated with menopause.[7,8,9]

The safety and efficacy of fezolinetant are under investigation and have not been established. There is no guarantee the agent will receive regulatory approval or become commercially available for the uses being investigated.

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.

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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