Press Release

Astellas Submits New Drug Application for Conditional Approval of Avacincaptad Pegol for Geographic Atrophy in Japan

- Ministry of Health, Labour and Welfare to evaluate ACP as potential first and only treatment for patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD) in Japan -

TOKYO, February 5, 2025 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Naoki Okamura, "Astellas") today announced the submission of a New Drug Application (NDA) to Japan's Ministry of Health, Labour and Welfare (MHLW) for Conditional Approval of avacincaptad pegol intravitreal solution (ACP), a synthetic aptamer that inhibits the complement C5 protein, for the treatment of GA secondary to AMD. If approved, ACP has the potential to become the first and only GA treatment available in Japan.

GA is a progressive form of AMD that can cause irreversible vision loss, with no treatments currently approved outside the US or Australia.[1,2] Globally, over five million people are estimated to have GA and, without timely treatment, an estimated 66% of people living with GA may become legally blind or severely visually impaired.[1,3] As a result, GA secondary to AMD has a substantial impact on patients' daily lives and psychological wellbeing.[4,5]

Marci English, Vice President, Head of BioPharma and Ophthalmology Development, Astellas
"Today's submission comes as good news to people in Japan living with geographic atrophy who have no approved treatment options for this devastating disease. If approved, avacincaptad pegol has the potential to be the first and only treatment to slow disease progression for eligible patients in Japan. As such, we are committed to working with regulatory authorities in Japan to ensure that patients can benefit from this vital new treatment."

The NDA submission is based on results of overseas clinical trials, including the GATHER1 and GATHER2 randomized, sham-controlled clinical trials, which evaluated the safety and efficacy of monthly 2mg intravitreal administration of ACP in patients with GA secondary to AMD.[6] The data from both trials demonstrates that ACP slows GA lesion growth and has a favorable safety profile.[6] Sustained efficacy of ACP, as shown in the restriction of lesion growth over time, was observed over a follow up of two years in GATHER1 and GATHER2 studies.[7]

This submission will have no impact on the financial forecasts of the current fiscal year ending March 31, 2025.

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About avacincaptad pegol

Avacincaptad pegol (ACP) is an investigational drug for treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) that has been submitted for Conditional Approval in Japan. ACP is approved in the U.S. as IZERVAY for the treatment of GA secondary to AMD.[6] ACP is a synthetic aptamer that inhibits the complement C5 protein.[8] Overactivity of the complement system and the C5 protein play a critical role in the development and growth of scarring and vision loss associated with GA secondary to AMD.[8] By targeting C5, ACP is considered to decrease activity of the complement system known to cause the degeneration of retinal cells and thus slow the progression of GA.[8]

About Geographic Atrophy

Age-related macular degeneration (AMD) is the major cause of moderate and severe loss of central vision in aging adults, affecting both eyes in the majority of patients.[1,8,9] The macula is a small area in the central portion of the retina responsible for central vision. As AMD progresses, the loss of retinal cells and the underlying blood vessels in the macula results in marked thinning and/or atrophy of retinal tissue.[10] Geographic atrophy (GA), associated with AMD, leads to further irreversible loss of vision in these patients.[11]

About the GATHER2 Clinical Trial

GATHER2 (NCT04435366) was a randomized, double-masked, sham-controlled, multicenter Phase 3 clinical trial to evaluate the safety and efficacy of intravitreal administration of avacincaptad pegol (ACP) in 448 enrolled patients with GA secondary to AMD.[6] ACP met its primary objective at 12 months, for which patients were randomized to receive either ACP or sham procedure monthly.[6] In year 2 of the study, patients treated with ACP in year 1 were re-randomized to receive either ACP dosed monthly (EM, n=96) or every other month (EOM, n=93); patients who received sham in year 1 continued to receive sham in year 2 (n=203).[7] IZERVAY is continuing to be evaluated in an open-label extension study.

About Astellas

Astellas is a global life sciences company committed to turning innovative science into VALUE for patients. We provide transformative therapies in disease areas that include oncology, ophthalmology, urology, immunology and women's health. Through our research and development programs, we are pioneering new healthcare solutions for diseases with high unmet medical need. Learn more at www.astellas.com.

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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