Iveric Bio Announces Submission of First Part of NDA for Rolling Review of Avacincaptad Pegol for the Treatment of Geographic Atrophy

November 3, 2022

PARSIPPANY, N.J.--(BUSINESS WIRE)--Nov. 3, 2022-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced that it has submitted to the U.S. Food and Drug Administration (FDA) the first part of its New Drug Application (NDA) for rolling review of avacincaptad pegol (ACP, also known as Zimura®) a novel investigational complement C5 inhibitor, for the treatment of geographic atrophy (GA) secondary to Age-Related Macular Degeneration (AMD). As previously announced, the Company received Fast Track designation from the FDA for ACP. Following receipt of the topline GATHER2 data, the Company shared the data with the FDA. Based on the pivotal clinical trials, GATHER1 and GATHER2, which both met their primary endpoint in slowing GA progression with statistical significance at the 12-month time point, the Company requested rolling submission of its planned NDA, which the FDA granted. As per the Company's agreement with the FDA, the first part of the NDA, which included the complete ACP clinical data package, was successfully submitted earlier today.

"We are pleased to report this important milestone and look forward to closely collaborating with the FDA throughout the review of our NDA," said Glenn P. Sblendorio, Chief Executive Officer of Iveric Bio. "As we stated previously, our Special Protocol Assessment (SPA) agreement with the FDA provides a basis for review of our NDA based on 12-month safety and efficacy results from GATHER2, taken together with the results of GATHER1."

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. 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. Geographic atrophy, the advanced stage of AMD, leads to further irreversible loss of vision in these patients. There are currently no U.S. FDA or European Medicines Agency (EMA) approved treatment options available for patients with geographic atrophy secondary to AMD.

About Avacincaptad Pegol
Avacincaptad pegol (ACP) is an investigational drug that has not yet been evaluated by any regulatory body for safety and efficacy. ACP is not authorized for any indication in any country. ACP is a novel complement C5 protein inhibitor. Overactivity of the complement system and the C5 protein are suspected to play a critical role in the development and growth of scarring and vision loss associated with geographic atrophy (GA) secondary to age-related macular degeneration (AMD). By targeting C5, ACP has the potential to decrease activity of the complement system that causes the degeneration of retinal cells and potentially slow the progression of GA.

About the GATHER Clinical Trials
The Company previously reported that ACP met its primary endpoint in its completed randomized, double-masked, sham-controlled, multicenter GATHER1 clinical trial and its ongoing GATHER2 clinical trial, both of which are Phase 3 randomized, double-masked, sham-controlled, multicenter clinical trials. These clinical trials measured the efficacy and safety of monthly 2 mg intravitreal administration of ACP in patients with GA secondary to AMD. For the first 12 months in both trials, patients were randomized to receive either ACP 2 mg or sham monthly. There were 286 participants enrolled in GATHER1 and 448 participants enrolled in GATHER2. The primary efficacy endpoints in both pivotal studies were based on GA area measured by fundus autofluorescence at three time points: Baseline, Month 6, and Month 12. The mean rate of growth (slope) in GA area from baseline to month 12 using observed data was 35% in GATHER 1 and 18% in GATHER2. In GATHER1 and GATHER2 combined, the most frequently reported treatment emergent adverse events in the 2 mg dose were related to injection procedure. The most common adverse reactions (≥ 5% and greater than sham) reported in patients who received avacincaptad pegol 2 mg were conjunctival hemorrhage (13%), increased IOP (9%), and CNV (7%). After 18 months of treatment in GATHER1 and 12 months of treatment in GATHER2, there were no events of serious intraocular inflammation, vasculitis, or endophthalmitis.

About FDA Fast Track

The FDA created the Fast Track process to facilitate the development and expedite the review of drugs to treat serious or life-threatening diseases or conditions, which have the potential to fill an unmet medical need. Fast Track designation offers important benefits, including frequent interactions with the FDA and the potential eligibility for Rolling Submission and Priority Review of a NDA, if relevant criteria are met. Currently, there is no FDA or European Medicines Agency (EMA) approved treatment option available for patients with GA secondary to AMD.

About Special Protocol Assessments

The SPA process is a procedure by which the FDA provides a clinical trial sponsor with an official evaluation and written guidance on the design of a proposed protocol intended to form the basis for a new drug application.

A SPA does not ensure the receipt of marketing approval or that the approval process will be faster than conventional regulatory procedures. Final marketing approval depends on efficacy and safety results and an evaluation of the overall benefits and risks of treatment after review of the data from the development program in its totality.

The SPA agreement may only be changed through a written agreement between the sponsor and the FDA, or if the FDA becomes aware of a substantial scientific issue essential to product efficacy or safety. For more information on Special Protocol Assessments, please visit: https://www.fda.gov/regulatory-information/search-fda-guidance-documents/special-protocol-assessment-guidance-industry.

About Iveric Bio

Iveric Bio is a science-driven biopharmaceutical company focused on the discovery and development of novel treatments for retinal diseases with significant unmet medical needs. The Company is committed to having a positive impact on patients' lives by delivering high-quality, safe and effective treatments designed to address debilitating retinal diseases including earlier stages of age-related macular degeneration. For more information on the Company, please visit www.ivericbio.com.

Forward-looking Statements

Any statements in this press release about the Company's future expectations, plans and prospects constitute forward-looking statements for purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995. Forward-looking statements include any statements about the Company's strategy, future operations and future expectations and plans and prospects for the Company, and any other statements containing the words "anticipate," "believe," "estimate," "expect," "intend", "goal," "may", "might," "plan," "predict," "project," "seek," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. In this press release, the Company's forward-looking statements include statements about its expectations regarding the results and implications of the clinical data from its GATHER1 and GATHER2 trial of ACP in geographic atrophy, its development and regulatory strategy for ACP, including its regulatory pathway and new drug application submission to the U.S. Food and Drug Administration, and the potential utility of ACP in treating geographic atrophy. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company's development programs, future results, performance, or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, those related to expectations for regulatory matters, interpretation of clinical trial results by the scientific and medical community, developments from the Company's competitors and the marketplace for the Company's products, and other factors discussed in the "Risk Factors" section contained in the quarterly and annual reports that the Company files with the Securities and Exchange Commission. Any forward-looking statements represent the Company's views only as of the date of this press release. The Company anticipates that subsequent events and developments may cause its views to change. While the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so except as required by law.

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