IVERIC bio Announces Publication of GATHER1 Phase 3 Clinical Trial Results for Zimura® in Geographic Atrophy Secondary to Age-related Macular Degeneration, in Ophthalmology®, the Journal of the American Academy of Ophthalmology

September 1, 2020

NEW YORK--(BUSINESS WIRE)--Sep. 1, 2020-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced that the positive Phase 3 results from its GATHER1 clinical trial with Zimura® (avacincaptad pegol), a novel complement C5 inhibitor, in development for the treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) were published in *Ophthalmology*®, the Journal of the American Academy of Ophthalmology. Zimura met its pre-specified primary efficacy endpoint at 12 months and reached statistical significance in GATHER1, which was an international, multicenter, randomized, double masked, sham controlled clinical trial. The reduction in the mean rate of GA growth over 12 months was 27.38% (p-value = 0.0072) for the Zimura 2 mg group as compared to the corresponding sham control group and 27.81% (p-value = 0.0051) for the Zimura 4 mg group as compared to the corresponding sham control group. The data for both dose groups were statistically significant. Zimura was generally well tolerated in the GATHER1 clinical trial.

The published article, entitled “C5 Inhibitor Avacincaptad Pegol for Geographic Atrophy Due to Age-Related Macular Degeneration: A Randomized Pivotal Phase 2/3 Trial,” is now available online at https://www.aaojournal.org/article/S0161-6420(20)30845-9/fulltext?dgcid=raven_jbs_aip_email.

“These published data bring Zimura one step closer as a potential treatment for patients with GA secondary to AMD, who currently do not have any treatment options available to them,” said Glenn Jaffe, M.D., lead author and Robert Machemer Professor of Ophthalmology, Chief of the Department of Ophthalmology Retina Division, Duke University. “Because Zimura inhibits C5 which is downstream in the complement cascade, upstream homeostatic complement activities including C3 are preserved. Zimura had a favorable safety profile. A treatment effect for the Zimura 2 mg and 4 mg groups was observed as early as 6 months. GATHER1 is currently the only Phase 3 clinical trial I am aware of showing early suppression of GA growth which continued throughout the trial with continuous treatment out to 18 months.”

“We are privileged to have the positive results of the Zimura GATHER1 clinical trial published in *Ophthalmology*, the highly-respected and peer-review publication of the American Academy of Ophthalmology,” stated Kourous A. Rezaei, M.D., Chief Medical Officer of IVERIC bio. “Our second Phase 3 clinical trial, GATHER2, is currently underway to further evaluate the efficacy and safety of Zimura in patients with geographic atrophy. If the primary efficacy endpoint is met at 12 months in the GATHER 2 trial, we plan to file for registration with the US and European regulatory authorities following receipt of that data. The absence of treatment options for geographic atrophy represents an area of urgent unmet medical need and a major public health concern for the expanding aging population.”

“We want to thank all the patients, clinical trial investigators and their staffs for their support and participation in this well conducted trial. We believe these robust, published, Phase 3 clinical data should further increase the enthusiasm for our ongoing GATHER2 clinical trial,” stated Glenn P. Sblendorio, Chief Executive Officer and President of IVERIC bio.

In the Company’s second Phase 3 clinical trial for Zimura in GA secondary to AMD, known as GATHER2, approximately 400 patients will be randomized to receive either monthly administration of Zimura 2 mg or sham during the first 12 months of the trial, at which time the primary efficacy analysis of the mean rate of change of GA growth at 12 months will be performed. At month 12, the Company plans to re-randomize patients in the Zimura 2 mg arm to receive either monthly or every other month administration of Zimura 2 mg. The final evaluation will take place at month 24.

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. Food and Drug Administration (FDA) or European Medicines Agency (EMA) approved treatment options available for patients with geographic atrophy.

Zimura
Complement factor C5 is a central component of the complement cascade and is believed to be involved in the development and progression of AMD. Zimura is designed to inhibit complement factor C5 cleavage into C5a and C5b. By inhibiting the formation of complement C5 terminal fragments, Zimura may decrease the activation of inflammasomes and the formation of membrane attack complex (MAC). This mechanism of action could potentially prevent or slow down the degeneration of retinal pigment epithelial (RPE) cells and slow down the progression of GA.

GATHER1 Clinical Trial
286 patients were enrolled in the GATHER1 clinical trial, with patients receiving Zimura 1 mg, Zimura 2 mg, Zimura 4 mg or a sham injection monthly for 18 months. Efficacy data from patients receiving Zimura 1 mg was not part of the prespecified primary statistical analysis.

In June 2018, IVERIC bio announced 18-month data from GATHER1 supporting the published 12 month data from this trial. The reduction in the mean rate of GA growth over 18 months was 28.11% for the Zimura 2 mg group as compared to the corresponding sham control group and 29.97% for the Zimura 4 mg group as compared to the corresponding sham control group. The pre-specified efficacy analysis for the primary endpoint was performed at month 12 using all of the power in the trial to detect a statistically significant difference. Therefore, the p-values for the 18 month statistical analyses are descriptive in nature. The descriptive p-values for the treatment effects at month 18 were p=0.0014 for the Zimura 2 mg group and p=0.0021 for the Zimura 4 mg group.

There was no Zimura-related inflammation, no Zimura-related discontinuations from the trial, no cases of endophthalmitis and no Zimura-related adverse events reported by investigators in the trial. Through month 18, the investigator reported incidence of choroidal neovascularization (CNV) in the untreated fellow eye was 11 patients (3.8%), and in the study eye was 3 patients (2.7%) in the sham control group, 2 patients (7.7%) in the Zimura 1 mg group, 8 patients (11.9%) in the Zimura 2 mg group, and 13 patients (15.7%) in the Zimura 4 mg group. The most frequently reported ocular adverse events were related to the injection procedure.

IVERIC bio
IVERIC bio is a science-driven biopharmaceutical company focused on the discovery and development of novel treatment options for retinal diseases with significant unmet medical needs. The Company is currently developing both therapeutic product candidates for age-related retinal diseases and gene therapy product candidates for orphan inherited retinal diseases. Vision is Our Mission. 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 the Company's expectations regarding its second Phase 3 trial (GATHER2) of Zimura in geographic atrophy secondary to AMD and to use its previously announced clinical trial of Zimura for the treatment of geographic atrophy (GATHER1) as a Phase 3 trial, its development and regulatory strategy for Zimura, the potential clinical meaningfulness of the results of clinical trials, the Company's hypotheses regarding complement inhibition as a mechanism of action for the treatment of geographic atrophy, the implementation of its business plan, the timing, progress and results of clinical trials and other research and development activities and regulatory submissions, and the potential utility of its product candidates. 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 the progression and duration of the COVID-19 pandemic and responsive measures thereto and related effects on the Company's research and development programs, operations and financial position, the initiation and the progress of research and development programs and clinical trials, availability of data from these programs, reliance on collaborators and other third parties, establishment of manufacturing capabilities, expectations for regulatory matters and need for additional financing 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 will 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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