Iveric Bio Reports Fourth Quarter and Year End 2020 Operational Highlights and Financial Results

March 3, 2021

- Patient Enrollment for GATHER2 Clinical Trial of Zimura® for the Treatment of Geographic Atrophy Secondary to Age-related Macular Degeneration Ahead of Target and Progressing Well; Completion of Enrollment Expected in 3Q of this Year -

- Conference Call and Webcast Today, March 3, 2021, at 8:00 a.m. ET -

NEW YORK--(BUSINESS WIRE)--Mar. 3, 2021-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced financial and operating results for the fourth quarter and full year ended December 31, 2020 and provided a general business update.

The Company also announced that patient enrollment and retention for GATHER2, its second Phase 3 clinical trial for Zimura® (avacincaptad pegol), a novel complement C5 inhibitor, for the treatment of geographic atrophy (GA) secondary to age-related macular degeneration (AMD) are progressing well with enrollment ahead of schedule. The Company is accelerating the timeline for when it expects to complete enrollment in GATHER2 to the third quarter of 2021. If the prespecified 12-month results from GATHER2 are positive, the Company plans to file applications with the results from GATHER1 and GATHER2 to the U.S. Food and Drug Administration and the European Medicines Agency for marketing approval of Zimura for GA.

“We start 2021 with significant momentum as we continue to enroll patients into our Zimura GATHER2 clinical trial and now expect to complete enrollment in the third quarter of this year,” stated Glenn P. Sblendorio, Chief Executive Officer and President of Iveric Bio. “We are extremely encouraged by the progress of our clinical trial sites to enroll and retain patients. We continue to work with our investigators to provide a safe environment for patients, which we believe increases the patients’ comfort and confidence to participate in the GATHER2 clinical trial. We are committed to continuing patient enrollment and retention aggressively in the GATHER2 clinical trial while prioritizing patient safety.”

Pravin U. Dugel, M.D., Chief Strategy and Business Officer of Iveric Bio added, “Although bringing Zimura to patients suffering from geographic atrophy secondary to age-related macular degeneration remains our top priority, in 2021 and thereafter we will continue to explore the potential development of Zimura in earlier stages of age-related macular degeneration as well as neovascular (wet) macular degeneration. Additionally, in our IC-500 program, we are pursuing HtrA1 inhibition as a target in the treatment of GA and potentially earlier stages of AMD. We are focused on advancing our pipeline of both therapeutic and gene therapy product candidates for treating retinal diseases with the potential to create long-term shareholder value.”

Therapeutics Programs Targeting Age-Related Macular Degeneration

Zimura® (avacincaptad pegol): Complement C5 Inhibitor

- In April 2020, the U.S. FDA granted Fast Track designation for Zimura for the treatment of GA secondary to dry AMD.
- In June 2020, the Company announced positive 18-month results from GATHER1, its first Phase 3 clinical trial for Zimura for the treatment of GA secondary to AMD. The 18-month data supports the previously announced 12-month data from this trial, at which time point Zimura met the pre-specified primary efficacy endpoint with statistical significance. Zimura was generally well tolerated after 18 months of administration.
- In late June 2020, the Company announced that the first patient had been dosed in the GATHER2 clinical trial.
- In September 2020, the Company announced that the positive 12-month Phase 3 results from its GATHER1 clinical trial with Zimura were published in *Ophthalmology*®, the Journal of the American Academy of Ophthalmology.
- In February 2021, Dr. Dugel presented the positive results from GATHER1 at the Angiogenesis, Exudation, Degeneration 2021 – Virtual Edition meeting.
- The Company increased the enrollment target in its ongoing Phase 2b screening clinical trial of Zimura for the treatment of autosomal recessive Stargardt disease. After initially enrolling 95 patients in this trial, the Company plans to enroll approximately 25 additional patients, with the goal of enrolling a total of 120 patients.

IC-500: HtrA1 (high temperature requirement A serine peptidase 1 protein) Inhibitor

- The Company recently revised its development plans for IC-500 to include plans to investigate multiple dosing schedules for this product candidate. Based on current timelines, the Company expects to submit an IND to the FDA for IC-500 in GA secondary to AMD in the second half of 2022.

Gene Therapy Programs in Orphan Inherited Retinal Diseases (IRDs)

- ***IC-100: Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa (RHO-adRP)***
  The Company is preparing an IND for IC-100 and plans to meet with regulatory authorities to discuss its selected doses for a first-in-human clinical trial prior to the submission. The Company plans to file an IND for IC-100 with the FDA and begin enrolling patients in a Phase 1/2 clinical trial for IC-100 in the second half of 2021.

- ***IC-200: BEST1-Related IRDs***
  The Company is completing IND-enabling activities for IC-200 and plans to file an IND for IC-200 with the FDA and begin enrolling patients in a Phase 1/2 clinical trial for IC-200 in the second half of 2021.

- ***Minigene Programs***
  The Company, in collaboration with the University of Massachusetts Medical School (UMMS), continues to advance its minigene programs for Leber Congenital Amaurosis Type 10 (LCA10), autosomal recessive Stargardt Disease (ABCA4), and USH2A-related IRDs. The Company expects to select a lead construct for its LCA10 minigene program in the second quarter of 2021. The Company expects to obtain additional results from its Stargardt Disease minigene program in the first half of 2021. The Company expects to obtain preliminary results from its USH2A minigene program in the first half 2021.

Corporate Highlights

During 2020, the Company expanded its Board of Directors and management by adding a number of leading industry experts. Mark S. Blumenkranz, M.D., M.M.S., HJ Smead Professor Emeritus, Department of Ophthalmology, Stanford University joined the Company's board of directors in July 2020. Pravin U. Dugel, M.D. joined the Company as Chief Strategy and Business Officer in March 2020. Dr. Dugel was previously Managing Partner, Retinal Consultants of Arizona and the Retinal Research Institute; Clinical Professor, USC Eye Institute, Keck School of Medicine, University of Southern California; and Founding Member, Spectra Eye Institute in Sun City, Arizona. Dhaval Desai, PharmD, joined the Company as Chief of Staff in August 2020. Previously, Mr. Desai served as Medical Unit Head at Novartis Eye Care.

In June 2020, the Company raised approximately $150 million in net proceeds in an underwritten public offering of common stock, and pre-funded warrants in lieu of common stock, and a concurrent private placement of common stock.

Fourth Quarter and Year End 2020 Operational Update and 2021 Cash Guidance

- As of December 31, 2020, the Company had $210 million in cash, cash equivalents and available for sale securities.

- The Company estimates its year-end 2021 cash, cash equivalents and available for sale securities will range between $130 and $140 million. The Company also estimates that its cash, cash equivalents and available for sale securities will be sufficient to fund its planned capital expenditure requirements and operating expenses, excluding any potential approval or sales milestones payable to Archemix Corp. or any commercialization expenses for Zimura, into 2024. These estimates are based on the Company's current business plan, including the continuation of its ongoing clinical development programs for Zimura, the progression of its IC-100 and IC-200 programs into the clinic, and the advancement of its IC-500 development program. These estimates also assume that the Company will enroll approximately 400 patients in the GATHER2 trial. These estimates do not reflect any additional expenditures related to potentially studying Zimura in other indications or resulting from the potential in-licensing or acquisition of additional product candidates or technologies or commencement of new sponsored research programs, and any associated development the Company may pursue.

2020 Financial Highlights

- **R&D Expenses**: Research and development expenses were $17.5 million for the quarter ended December 31, 2020, compared to $11.6 million for the same period in 2019. For the year ended December 31, 2020, research and development expenses were $62.8 million compared to $39.6 million for 2019. Research and development expenses increased primarily due to the initiation of GATHER2 and increased manufacturing activities for Zimura, increased manufacturing and preclinical development activities associated with the Company's IC-100 and IC-200 gene therapy programs and the progression of its IC-500 development program.

- **G&A Expenses**: General and administrative expenses were $8.0 million for the quarter ended December 31, 2020, compared to $6.3 million for the same period in 2019. For the year ended December 31, 2020 general and administrative expenses were $26.0 million compared to $21.6 million for 2019. General and administration expenses increased primarily due to increases in general consulting costs and professional fees.

- **Income Tax (Benefit)**: Income tax benefits of $3.7 million and $0.1 million for the years ended December 31, 2020 and 2019, respectively, were recognized to reflect the favorable settlement of local tax audits.

- **Net Income**: The Company reported a net loss for the quarter ended December 31, 2020 of $25.4 million, or ($0.27) per diluted share, compared to a net loss of $17.5 million, or ($0.39) per diluted share, for the same period in 2019. For the year ended December 31, 2020, the Company reported a net loss of $84.5 million or ($1.14) per diluted share, compared to a net loss of $58.9 million or ($1.39) for the same period in 2019.

Conference Call/Web Cast Information

Iveric Bio will host a conference call/webcast to discuss the Company's financial and operating results and provide a business update. The call is scheduled for March 3, 2021 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 888-221-3881 (USA) or 323-794-2590 (International), passcode 9597743. A live, listen-only audio webcast of the conference call can be accessed on the Investors section of the Iveric Bio website at www.ivericbio.com. A replay will be available approximately two hours following the live call for two weeks. The replay number is 888-203-1112 (USA), passcode 9597743.

About 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 its expectations regarding patient enrollment and patient retention in its second Phase 3 trial (GATHER2) of Zimura in geographic atrophy secondary to AMD and use of the results from its completed clinical trial of Zimura for the treatment of geographic atrophy secondary to AMD (GATHER1) as a Phase 3 trial, its development and regulatory strategy for Zimura and its other product candidates, including additional indications that the Company may pursue for the development of Zimura and IC-500, the implementation of its business plan, its expectations regarding expected cash, cash equivalents and available for sale securities and the sufficiency of its cash resources, the timing, progress and results of clinical trials and other research and development activities and regulatory submissions, the potential utility of its product candidates, the potential for its business development strategy and its personnel and human capital resources. 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, including enrollment and retention in clinical trials, availability of data from these programs, reliance on contract development and manufacturing organizations, university collaborators and other third parties, establishment of manufacturing capabilities, expectations for regulatory matters, developments from the Company's competitors and the marketplace for the Company's products, need for additional financing and negotiation and consummation of business development transactions 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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IVERIC bio, Inc.
Selected Financial Data (unaudited)
(in thousands, except per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2020	**2019**	**2020**	**2019**
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 17,473	$ 11,567	$ 62,784	$ 39,644
General and administrative	8,022	6,275	25,952	21,628
Total operating expenses	25,495	17,842	88,736	61,272
Loss from operations	(25,495)	(17,842)	(88,736)	(61,272)
Interest income	63	369	500	2,151
Other income (expense), net	-	-	(6)	151
Loss before income tax provision (benefit)	(25,432)	(17,473)	(88,242)	(58,970)
Income tax provision (benefit)	-	5	(3,695)	(111)
Net loss	$ (25,432)	$ (17,478)	$ (84,547)	$ (58,859)
Net loss per common share:				
Basic and diluted	$ (0.27)	$ (0.39)	$ (1.14)	$ (1.39)
Weighted average common shares outstanding:				
Basic and diluted	92,810	44,413	74,185	42,224

	December 31, 2020	December 31, 2019

	(in thousands)	
Balance Sheets Data:		
Cash, cash equivalents and marketable securities	$ 210,047	$ 125,699
Total assets	$ 216,754	$ 130,187
Total liabilities	$ 25,191	$ 12,984
Additional paid-in capital	$ 756,543	$ 597,679
Accumulated deficit	$ (565,073)	$ (480,526)
Total stockholders' equity	$ 191,563	$ 117,203

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