Iveric Bio Reports Fourth Quarter and Year End 2019 Operational Highlights and Financial Results

February 27, 2020

- Conference Call and Webcast Today, February 27, 2020, at 8:00 a.m. ET -

NEW YORK--(BUSINESS WIRE)--Feb. 27, 2020-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced financial and operating results for the fourth quarter and full year ended December 31, 2019 and provided a general business update.

"We made tremendous progress last year as we build a diversified portfolio in retinal diseases that includes both therapeutics and gene therapy, setting the stage for IVERIC bio to be a leader in developing transformative therapies to treat retinal diseases," stated Glenn P. Sblendorio, Chief Executive Officer and President of IVERIC bio. "We achieved a major milestone with our positive Zimura pivotal clinical trial results in geographic atrophy secondary to dry AMD. Our goal is to continue to build on this momentum. Following the positive data, our team quickly started working on our second Zimura pivotal clinical trial in GA with plans to enroll the first patient next month. Our lead gene therapy programs in rhodopsin mediated adRP and BEST1 related retinal diseases continue to advance towards Phase 1/2 clinical trials and we expect to identify our lead minigene construct for LCA10 later in the year."

Therapeutics Programs

Zimura® (avacincaptad pegol): Complement C5 Inhibitor

- On October 28, 2019, the Company announced that Zimura® (avacincaptad pegol) met its pre-specified primary efficacy endpoint and reached statistical significance in an international, multicenter, randomized, double masked, sham controlled clinical trial in geographic atrophy (GA) secondary to dry age-related macular degeneration (AMD), referred to as the OPH2003 trial. Zimura was generally well tolerated after 12 months of administration. The Company believes that the safety and efficacy results from this trial could meet regulatory agencies' requirements to serve as one of the two pivotal clinical trials typically required for marketing approval.
- In January 2020, the Company announced the design of its second pivotal clinical trial of Zimura in GA secondary to dry AMD, ISEE2008. The Company plans to enroll approximately 400 patients in this international, multicenter, double masked, sham controlled clinical trial. Patients will be randomized to receive either monthly administration of Zimura 2mg 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. If the 12 month results are positive, the Company plans to file an application with the U.S. Food and Drug Administration and the European Medicines Agency for marketing approval of Zimura for GA following receipt of that data. 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. All the patients who were initially randomized to the sham control arm will continue with monthly administration of sham. The final evaluation will take place at month 24.
- The Company plans and is on track to enroll the first patient in the ISEE2008 trial next month.
- The Company's ongoing Phase 2b clinical trial of Zimura for the treatment of autosomal recessive Stargardt disease, an orphan inherited retinal disease, is on track for top-line data to be available during the second half of 2020.

HtrA1 Inhibitor

- The Company has identified a lead compound in this preclinical program to address GA secondary to dry AMD and is developing the formulation and manufacturing process with the goal of filing an IND during 2021.

Gene Therapy Programs in Orphan Inherited Retinal Diseases

- ***IC-100: Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa (RHO-adRP)***
  Natural history studies and IND-enabling activities for IC-100 are ongoing. The Company plans to initiate a Phase 1/2 clinical trial for IC-100 in patients with rhodopsin mediated adRP during the fourth quarter of 2020.
- ***IC-200: BEST1-Related IRDs***
  Natural history studies and IND-enabling activities for IC-200 are ongoing. The Company plans to initiate a Phase 1/2 clinical trial for IC-200 in patients with BEST1 related retinal diseases during the first half of 2021.
- ***miniCEP290: Leber Congenital Amaurosis Type 10 (LCA10)***
  IVERIC bio, in collaboration with the University of Massachusetts Medical School (UMass Medical School), is continuing to optimize the minigene constructs with the goal of identifying a lead construct by mid-year 2020.
- ***miniABCA4 Program for Stargardt Disease (STGD1)***

IVERIC bio, through its collaborative sponsored research agreement with UMass Medical School, is evaluating several ABCA4 minigene constructs in both in vitro and in vivo experiments. The Company has received preliminary results and expects to receive additional results for the miniABCA4 program during the second half of 2020.

- ***miniUSH2A: USH2A-Related IRDs Including Usher Syndrome Type 2A (Usher 2A) and USH2A-Associated Nonsyndromatic Autosomal Recessive Retinitis Pigmentosa***
  This research program targets IRDs associated with mutations in the USH2A gene, including Usher 2A and USH2A-associated nonsyndromatic autosomal recessive retinitis pigmentosa. The Company expects to receive preliminary results during the second half of 2020.

Corporate Update

In December 2019, the Company completed an underwritten public offering in which it sold 7,750,000 shares of its common stock at a price of $4.00 per share, and it also sold to certain investors pre-funded warrants to purchase 3,750,000 shares of its common stock at a price of $3.999 per share underlying each warrant. The Company raised approximately $42.6 million in net proceeds from this offering.

During the fourth quarter of 2019, IVERIC bio appointed Guangping Gao, PhD as Chief Strategist, Gene Therapy, and Abraham Scaria, PhD as Chief Scientific Officer.

Fourth Quarter and Year End 2019 Financial Results and 2020 Cash Guidance

As of December 31, 2019, the Company had $125.7 million in cash and cash equivalents. The Company estimates that its year-end 2020 cash and cash equivalents will range between $60 million and $70 million. The Company also estimates that its cash and cash equivalents will be sufficient to fund its operations and capital expenditure requirements as currently planned into the beginning of 2022. These estimates are based on the Company's current business plan, including initiation of the Zimura ISEE2008 trial and the continuation of the Company's other on-going research and development programs. These estimates do not reflect any additional expenditures, including associated development costs, in the event the Company in-licenses or acquires any new product candidates or commences any new sponsored research programs.

2019 Financial Highlights

- **R&D Expenses**: Research and development expenses were $11.6 million for the quarter ended December 31, 2019, compared to $16.1 million for the same period in 2018. For the year ended December 31, 2019, research and development expenses were $39.6 million compared to $41.7 million for the same period in 2018. Research and development expenses decreased primarily due to decreased costs associated with the Company's Zimura OPH2003 clinical trial in GA and OPH2005 clinical trial in Stargardt disease, as earlier-enrolled patients completed those trials, and decreased costs related to the Company's acquisition of Inception 4 and its HtrA1 inhibitor program during 2018. These decreases were offset by increases in costs associated with the Company's gene therapy programs.
- **G&A Expenses**: General and administrative expenses were $6.3 million for the quarter ended December 31, 2019, compared to $5.7 million for the same period in 2018. For the year ended December 31, 2019, general and administrative expenses were $21.6 million compared to $23.6 million for the same period in 2018. General and administrative expenses decreased primarily due to decreases in costs to support the Company's operations and infrastructure.
- **Net Income (loss)**: The Company reported a net loss for the quarter ended December 31, 2019 of $17.5 million, or ($0.39) per diluted share, compared to a net income of $104.1 million, or $2.62 per diluted share, for the same period in 2018. In the quarter and year ended December 31, 2018, the Company recognized a gain on extinguishment of a royalty purchase liability of $125 million due to its December 2018 termination of a royalty and sales agreement with Novo Holdings A/S. For the year ended December 31, 2019, the Company reported a net loss of $58.9 million or ($1.39) per diluted share, compared to a net income of $63.1 million or $1.70 for the same period in 2018.

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 February 27, 2020 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 800-458-4121 (USA) or 323-794-2598 (International), passcode 6010573. 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 Toll Free), passcode 6010573.

About IVERIC bio

IVERIC bio is a biopharmaceutical company focused on the discovery and development of novel treatment options for retinal diseases with significant unmet medical needs. 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 to use its previously announced clinical trial of Zimura for the treatment of geographic atrophy as a pivotal trial, its development strategy for Zimura, including its plans and expectations regarding a second, pivotal clinical trial evaluating Zimura for the treatment of

geographic atrophy, the implementation of its business plan, the projected use of cash and cash balances, the timing, progress and results of clinical trials and other research and development activities, the potential utility of its product candidates, and the potential for its business development strategy. 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 initiation and the progress of research and development programs and clinical trials, availability of data from these programs, reliance on university collaborators and other third parties, establishment of manufacturing capabilities, expectations for regulatory matters, 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,	
	2019	**2018**	**2019**	**2018**
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 11,567	$ 16,128	$ 39,644	$ 41,737
General and administrative	6,275	5,667	21,628	23,612
Total operating expenses	17,842	21,795	61,272	65,349
Loss from operations	(17,842)	(21,795)	(61,272)	(65,349)
Interest income	369	677	2,151	2,389
Gain on extinguishment of Royalty Purchase Liability	-	125,000	-	125,000
Other income (expense)	-	1	151	(16)
Income (loss) before income tax provision (benefit)	(17,473)	103,883	(58,970)	62,024
Income tax provision (benefit)	5	(231)	(111)	(1,063)
Net Income (loss)	$ (17,478)	$ 104,114	$ (58,859)	$ 63,087
Net loss per common share:				
Basic	$ (0.39)	$ 2.62	$ (1.39)	$ 1.70
Diluted	$ (0.39)	$ 2.62	$ (1.39)	$ 1.70
Weighted average common shares outstanding:				
Basic	$ 44,413	$ 39,673	$ 42,224	$ 37,061
Diluted	44,413	39,687	42,224	37,088

	December 31, 2019	December 31, 2018
	(in thousands)	
Balance Sheets Data:		
Cash and cash equivalents	$ 125,699	$ 131,201
Total assets	130,187	137,165
Total liabilities	12,984	13,206
Additional paid-in capital	597,679	545,585
Accumulated deficit	(480,526)	(421,667)
Total stockholders' equity	$ 117,203	$ 123,959

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