# Iveric Bio Reports Second Quarter 2021 Operational Highlights and Financial Results

August 4, 2021

*- FDA Agreement Under Special Protocol Assessment (SPA) Received for GATHER2 Phase 3 Clinical Trial of Zimura® in Geographic Atrophy Secondary to Age-Related Macular Degeneration –*

*- Zimura GATHER2 Enrollment Complete; Retention Exceeding Expectations, with Injection Fidelity Rate Target of Greater than 90% -*

*- GATHER1 18 Month Post-Hoc Analyses Show Zimura 2 mg Has the Potential to Have an Impact on Earlier Stages of Dry AMD Prior to Geographic Atrophy -*

*- Conference Call and Webcast Today, August 4, 2021, at 8:00 a.m. ET -*

NEW YORK--(BUSINESS WIRE)--Aug. 4, 2021-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced financial and operating results for the quarter ended June 30, 2021 and provided a general business update.

“This is an exciting time for the Company as we have delivered on several transformational milestones, particularly with regard to the execution of our Zimura pivotal program,” stated Glenn P. Sblendorio, Chief Executive Officer of Iveric Bio. “For GATHER2, our second Zimura Phase 3 clinical trial for geographic atrophy secondary to age-related macular degeneration, we received a Special Protocol Assessment agreement from the FDA, we completed enrollment four months ahead of schedule, and patient retention, as measured by the injection fidelity rate, continues to exceed expectations and we are targeting the 12-month rate to be greater than 90%. In addition, the results of post-hoc analyses from GATHER1, our first Zimura Phase 3 clinical trial in GA secondary to AMD, indicate that Zimura may have a therapeutic benefit in earlier stages of GA.”

“We are extremely proud of the momentum that we have created,” stated Pravin U. Dugel, President of Iveric Bio. “During July 2021, we strengthened our balance sheet with a public offering. We believe this enables us to further support the impressive recruitment with superb retention in our Zimura GATHER2 Phase 3 trial, prepare and potentially file a New Drug Application for Zimura in GA secondary to AMD, begin preparations for a potential commercial launch of Zimura in GA secondary to AMD, initiate a drusen clinical development program, and invest in sustained release delivery technologies for Zimura. We are committed to execute on our strategy to develop and deliver treatments for diseases of the retina through our Zimura, HtrA1 inhibitor and gene therapy programs, with the potential to create long-term shareholder value.”

**Therapeutics Programs Targeting Geographic Atrophy (GA) Secondary to Age-Related Macular Degeneration (AMD)**

***Zimura® (avacincaptad pegol): Complement C5 Inhibitor***

- ***Special Protocol Assessment (SPA) for GATHER2***
  The Company announced on July 6, 2021, that it received written agreement from the U.S. Food and Drug Administration (FDA) under a Special Protocol Assessment (SPA) for the overall design of GATHER2, the Company’s second pivotal clinical trial of Zimura in development for the treatment of GA secondary to AMD. The agreement further solidifies the Company’s plans to file an application with the FDA for marketing approval of Zimura for GA secondary to AMD, if the ongoing GATHER2 clinical trial meets its primary endpoint at 12 months. Zimura met its pre-specified primary efficacy endpoint at 12 months and reached statistical significance in the previously completed GATHER1 pivotal clinical trial.

  In parallel discussions with those for the GATHER2 SPA, the FDA indicated to the Company that, as part of a future NDA submission for Zimura, the GATHER1 results will be considered using the original prespecified primary efficacy endpoint analysis, together with a post-hoc analysis using the same FDA preferred method that will be used for the GATHER2 trial (mean rate of growth (slope) estimated based on GA area measured by fundus autofluorescence (FAF) in the relevant timepoints). The GATHER 1 results as analyzed by the FDA preferred analysis is highly consistent with and strongly supportive of the results from the original prespecified analysis. The complete results of both analyses for GATHER1 have been presented in the Company’s July 6, 2021 press release and Form 8-K filing.

- ***GATHER2 Enrollment, Retention, and Injection Fidelity Rate***
  On July 26, 2021, the Company announced completion of enrollment in GATHER2, four months ahead of the Company’s original schedule. Based on this timeline, the Company expects topline GATHER2 data to be available in the second half of 2022, approximately one year after the enrollment of the last patient plus the time needed for database lock and analysis.

  In June 2021, the Company announced that it is targeting patient retention for the GATHER2 trial, as measured by injection fidelity rate through month 12, of greater than 90%. The injection fidelity rate continues to exceed the Company’s

expectations. Injection fidelity is calculated by dividing the total number of actual injections by the total number of expected injections based on the number of enrolled patients. The Company considers injection fidelity to be the most important and stringent measure of patient retention because it reflects the timely administration of the drug into the patient's eye.

- ***Earlier Stages of Dry AMD Prior to Geographic Atrophy***
  In June 2021, Vas Sadda, MD, of Doheny Eye Institute at UCLA, presented new post-hoc analyses from the GATHER1 trial on progression of drusen and nascent GA (iRORA/cRORA), which are earlier forms of dry AMD, in patients treated with Zimura 2 mg as compared to patients in the sham group. These post-hoc analyses suggest that Zimura may have the potential to impact AMD even before atrophy occurs, thereby changing the natural course of the disease. The Company expects to initiate a drusen clinical development program in 2022.

- ***Autosomal Recessive Stargardt Disease***
  Patient enrollment in the Phase 2b screening clinical trial of Zimura for the treatment of autosomal recessive Stargardt disease, referred to as the STAR trial, is ongoing with the goal of enrolling approximately 25 new patients for a total of approximately 120 patients.

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

During the second quarter of 2021, the Company commenced its first preclinical tolerability study for IC-500 and is currently planning additional preclinical studies, including pharmacokinetic and target engagement studies. Formulation optimization and other manufacturing activities are also ongoing. The Company expects to submit an investigational new drug application (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-200: BEST1-Related IRDs***
  The Company has completed an IND-enabling preclinical toxicology study of IC-200, in a naturally occurring canine disease model of Best disease. Subject to regulatory review, the Company plans to initiate a Phase 1/2 clinical trial of IC-200 during the fourth quarter of 2021. The first IC-200 clinical trial will focus on patients with the autosomal recessive form of the disease, autosomal recessive bestrophinopathy.

- ***IC-100: Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa (RHO-adRP)***
  As previously disclosed, the Company was planning to discuss with the FDA the results from its toxicology studies of IC-100, and the design of its first-in-human clinical trial, before submitting an IND. The FDA advised, in lieu of this meeting, additional discussion should be conducted during the 30-day IND review period following IND submission. The Company is currently considering its development options for this product candidate.

- ***Minigene Programs***
  The Company, through its minigene collaboration with the University of Massachusetts Medical School (UMMS), has identified a lead construct for its Leber Congenital Amaurosis Type 10 (LCA10) miniCEP290 program and is currently planning preclinical development for this program. The Company is evaluating preclinical data from its Stargardt Disease (ABCA4) program and expects to obtain preliminary results from its USH2A-related inherited retinal diseases (USH2A) program in the first half of 2022.

  The Company recently hired four individuals who were previously at UMMS, including Hemant Khanna, Ph.D., the principal investigator for the Company's miniCEP290, miniABCA4 and miniUSH2A sponsored research programs. Dr. Khanna joined the Company as Vice President, Pre-Clinical Ocular Research. The Company is working to transition the research and preclinical development activities for these programs from UMMS to the Company. The Company is also preparing to establish laboratory space for these employees to continue working on these programs and other preclinical research and development activities for the Company.

**Management Updates**

- In August 2021, Christopher Simms joined Iveric Bio as Senior Vice President and Chief Commercial Officer. Mr. Simms is an accomplished healthcare leader with more than 20 years of diverse commercial leadership experience at Johnson & Johnson, Genentech, and Novartis, including focused experience in retina, ophthalmology, and optometry.

**Second Quarter Financial Results and 2021 Cash Guidance**

- As of June 30, 2021, the Company had $159.9 million in cash and cash equivalents.

- In July 2021, the Company raised approximately $108 million in net proceeds in an underwritten public offering of common stock. The Company sold 13,397,500 shares of its common stock in this public offering.

- The Company now estimates its year-end 2021 cash, cash equivalents and available for sale securities to range between

$215 million and $225 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 through at least mid-year 2024. These estimates are based on the Company's current business plan including the continuation of its ongoing clinical development programs for Zimura, preparation and potential filing of a New Drug Application and a Marketing Authorization Application for Zimura in GA secondary to AMD, beginning preparations for a potential commercial launch of Zimura in GA secondary to AMD, initiating a drusen clinical development program, and investing in sustained release delivery technologies for Zimura, the progression of its IC-200 and IC-100 programs into the clinic, and the advancement of its IC-500 development program. Excluded from these estimates are any potential approval or sales milestones payable to Archemix Corp. and potential expenses for actual commercial launch of Zimura, any additional expenditures related to potentially studying Zimura in indications outside of GA and drusen or resulting from the potential in-licensing or acquisition of additional product candidates or technologies, and any associated development the Company may pursue.

**2021 Q2 Financial Highlights**

- **R&D Expenses**: Research and development expenses were $23.5 million for the quarter ended June 30, 2021, compared to $12.7 million for the same period in 2020. For the six months ended June 30, 2021, research and development expenses were $42.0 million compared to $26.5 million for the same period in 2020. Research and development expenses increased primarily due to the commencement of patient enrollment and ongoing progress of the GATHER2 clinical trial, increased manufacturing activities for Zimura and increases in research and development personnel.

- **G&A Expenses**: General and administrative expenses were $6.7 million for the quarter ended June 30, 2021, compared to $6.3 million for the same period in 2020. For the six months ended June 30, 2021, general and administration expenses were $15.0 million compared to $11.3 million for the same period in 2020. General and administration expenses increased primarily due to legal costs associated with continued litigation efforts.

- **Income Tax Benefit**: The Company recorded no income tax benefit for the three and six months ended June 30, 2021. Income tax benefits of $0.4 million and $3.7 million for the three and six months ended June 30, 2020, respectively, were recognized to reflect a favorable settlement of a state corporate income tax audit.

- **Net Loss**: The Company reported a net loss for the quarter ended June 30, 2021 of $30.1 million, or ($0.32) per diluted share, compared to a net loss of $18.6 million, or $(0.32) per diluted share, for the same period in 2020. For the six months ended June 30, 2021, the Company reported a net loss of $56.9 million or ($0.61) per diluted share, compared to a net loss of $33.7 million or ($0.61) for the same period in 2020.

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 August 4, 2021 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 1-888-317-6003 (USA) or 1-412-317-6061 (International), passcode 7257034 . 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 1-877-344-7529 (USA) or 1-412-317-0088 , passcode 10158843.

**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. 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 availability of topline data from 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 its plans to submit a new drug application to the U.S. Food and Drug Administration and a marketing authorization application to the European Medicines Agency for Zimura if the results from GATHER2 are positive and its plans to initiate clinical development in drusen, the implementation of its business plan, its projected use of 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, expectations for regulatory matters, 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 clinical trial sites, contract development and manufacturing organizations, university collaborators and other third parties, establishment of manufacturing capabilities, developments from the Company's competitors and the marketplace for the Company's products, human capital 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 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.*

**IVERIC bio, Inc.**
**Selected Financial Data (unaudited)**
**(in thousands, except per share data)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | **2021** | **2020** | **2021** | **2020** |
| **Statements of Operations Data:** | | | | |
| Operating expenses: | | | | |
| Research and development | $ 23,488 | $ 12,720 | $ 42,037 | $ 26,470 |
| General and administrative | 6,718 | 6,289 | 15,040 | 11,287 |
| Total operating expenses | 30,206 | 19,009 | 57,077 | 37,757 |
| Loss from operations | (30,206) | (19,009) | (57,077) | (37,757) |
| Interest income | 65 | 46 | 142 | 404 |
| Other income (expense), net | (2) | (12) | (3) | (7) |
| Loss before income benefit | (30,143) | (18,975) | (56,938) | (37,360) |
| Income tax benefit | - | 386 | - | 3,695 |
| Net loss | $ (30,143) | $ (18,589) | $ (56,938) | $ (33,665) |
| Net loss per common share: | | | | |
| Basic and diluted | $ (0.32) | $ (0.32) | $ (0.61) | $ (0.61) |
| Weighted average common shares outstanding: | | | | |
| Basic and diluted | 93,409 | 57,421 | 93,382 | 55,424 |

| | **June 30, 2021** | **December 31, 2020** |
|---|---|---|
| | **(in thousands)** | |
| **Balance Sheets Data:** | | |
| Cash, cash equivalents and marketable securities | $ 159,882 | $ 210,047 |
| Total assets | $ 165,667 | $ 216,754 |
| Total liabilities | $ 26,094 | $ 25,191 |
| Additional paid-in capital | $ 761,491 | $ 756,543 |
| Accumulated deficit | $ (622,011) | $ (565,073) |
| Total stockholders' equity | $ 139,573 | $ 191,563 |

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