IVERIC bio Reports Second Quarter 2019 Financial and Operating Results

August 1, 2019

- Company Continues to Expand its Gene Therapy Portfolio with the Addition of miniUSH2A Program -

- Company to Host First Gene Therapy R&D Investor Day on September 13, 2019 -

- Conference Call and Webcast Today, August 1, 2019, at 8:00 a.m. ET -

NEW YORK--(BUSINESS WIRE)--Aug. 1, 2019-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced financial and operating results for the second quarter ended June 30, 2019 and provided a business update.

“We are pleased with the Company’s progress as we achieved several milestones to advance and expand our gene therapy pipeline for orphan inherited retinal diseases, or IRDs,” stated Glenn P. Sblendorio, Chief Executive Officer and President of IVERIC bio. “We entered into exclusive global license agreements for rights to gene therapy product candidates for the treatment of *BEST1*-related IRDs and Leber congenital amaurosis type 10, or LCA10. We are encouraged by new research data from our miniCEP290 collaboration that support our plans to move this program forward in LCA10, and we expanded our gene therapy portfolio with the addition of a minigene research program for *USH2A* related IRDs, including Usher syndrome type 2A and *USH2A*-associated nonsyndromatic autosomal recessive retinitis pigmentosa. Manufacturing activities for IC-100 and IC-200, our two lead gene therapy product candidates, are currently ongoing using state-of-the-art manufacturing capabilities of Paragon Gene Therapy, part of Catalent Biologics.”

Kourous A. Rezaei, MD, Chief Medical Officer of IVERIC bio added, “With the addition of the *USH2A* program to our portfolio, IVERIC bio is seeking to treat vision loss associated with what are reportedly the most common form of autosomal dominant retinitis pigmentosa (rhodopsin-mediated) and the most common form of autosomal recessive retinitis pigmentosa (USH2A-related). Retinitis pigmentosa is the most common orphan IRD. We look forward to presenting our gene therapy pipeline and discussing our programs in greater detail with highly-recognized gene therapy scientists and key opinion leaders in retinal diseases at our upcoming Gene Therapy R&D Investor Day.”

Second Quarter 2019 and Recent Highlights

Orphan IRD Gene Therapy Programs

- ***IC-100: Rhodopsin-Mediated Autosomal Dominant Retinitis Pigmentosa (RHO-adRP)***

Natural history studies and IND-enabling activities for IC-100 are ongoing. The Company expects to initiate a Phase 1/2 clinical trial for IC-100 in the second half of 2020.

- ***IC-200: BEST1-Related IRDs***

In April 2019, the Company entered into an exclusive global license agreement with the University of Pennsylvania and the University of Florida Research Foundation for rights to develop and commercialize novel adeno-associated virus (AAV) gene therapy product candidates for the treatment of *BEST1*-related IRDs, including Best vitelliform macular dystrophy, also known as Best disease. The Company expects to initiate a Phase 1/2 clinical trial for IC-200 in the first half of 2021.

- ***miniCEP290: Leber Congenital Amaurosis Type 10 (LCA10)***

Encouraging results from the Company’s collaboration with the University of Massachusetts Medical School (UMass Medical School) in its miniCEP290 program led the Company to exercise its option and, in July 2019, the Company entered into an exclusive global license agreement with the University of Massachusetts for rights to develop and commercialize mutation independent novel AAV minigene therapy product candidates for the treatment of LCA10, which is due to mutations in the *CEP290* gene and is the most common type of LCA.

- ***miniUSH2A: USH2A-Related IRDs Including Usher Syndrome Type 2A and USH2A-Associated Nonsyndromatic Autosomal Recessive Retinitis Pigmentosa***

In July 2019, the Company entered into a sponsored research agreement with UMass Medical School and an exclusive option agreement with the University of Massachusetts for rights to develop and commercialize novel AAV gene therapy product candidates utilizing a mutation independent minigene therapy approach for the treatment of *USH2A*-related IRDs. This group of orphan IRDs include Usher syndrome Type 2A and *USH2A*-associated nonsyndromatic autosomal recessive retinitis pigmentosa.

Gene Therapy Manufacturing

In June 2019, the Company announced that it had entered into a strategic manufacturing relationship with Paragon Gene Therapy, part of Catalent Biologics. This agreement is for production and manufacturing of AAV vectors for pre-clinical activities and planned Phase 1/2 clinical trials for IC-100 and IC-200.

Gene Therapy R&D Investor Day Scheduled

The Company will host its first “Gene Therapy R&D Investor Day” in New York, NY on Friday, September 13, 2019. The agenda includes updates from IVERIC bio’s management on its gene therapy portfolio in orphan IRDs, and scientific discussions from gene therapy scientists and key opinion leaders in orphan inherited retinal diseases including;

- Guangping Gao, PhD, University of Massachusetts Medical School
- William A. Beltran, DVM, PhD, University of Pennsylvania
- Charles A. Gersbach, Duke University
- Hemant Khanna, PhD, University of Massachusetts Medical School
- Gustavo D. Aguirre, VMD, PhD, University of Pennsylvania School of Veterinary Medicine
- Andreas K. Lauer, MD, Oregon Health & Science University
- Bart P. Leroy, MD, PhD, Ghent University and Children’s Hospital of Philadelphia
- Alfred S. Lewin, PhD, University of Florida

Therapeutic Programs

The Company expects initial top-line data for its ongoing Phase 2b clinical trial of Zimura® (avacincaptad pegol), a C5 complement inhibitor, for the treatment of geographic atrophy secondary to dry age-related macular degeneration to be available in the fourth quarter of 2019. The Company expects initial top-line data for its Phase 2b clinical trial of Zimura for the treatment of autosomal recessive Stargardt disease to be available in the second half of 2020.

Second Quarter 2019 Financial Results

Operational Update

As of June 30, 2019, the Company had $106.9 million in cash and cash equivalents. After taking into account the Company’s recent in-licensing transaction for its miniCEP290 program and sponsored research agreement for its miniUSH2A program, the Company reaffirms its estimate that year-end 2019 cash and cash equivalents will range between $80 million and $85 million. This estimate is based on its current 2019 business plan, including the continuation of its current research and development programs. This estimate does 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.

- **R&D Expenses**: Research and development expenses were $10.0 million for the quarter ended June 30, 2019, compared to $8.5 million for the same period in 2018. For the six months ended June 30, 2019, research and development expenses were $17.7 million compared to $16.2 million for the same period in 2018. Research and development expenses increased primarily due to increases in costs associated with the Company’s gene therapy programs and HtrA1 inhibitor program, offset by decreases in costs associated with the Company’s Zimura programs, personnel expenses and professional and consulting fees.

- **G&A Expenses**: General and administrative expenses were $5.2 million for the quarter ended June 30, 2019, compared to $6.3 million for the same period in 2018. For the six months ended June 30, 2019, general and administrative expenses were $10.7 million compared to $12.0 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**: The Company reported a net loss for the quarter ended June 30, 2019 of $14.4 million, or ($0.35) per diluted share, compared to a net loss of $13.2 million, or ($0.37) per diluted share, for the same period in 2018. For the six months ended June 30, 2019, the Company reported a net loss of $26.9 million or ($0.65) per diluted share, compared to a net loss of $26.3 million or ($0.73) 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 August 1, 2019 at 8:00 a.m. Eastern Time. To participate in this conference call, dial 800-458-4121 (USA) or 323-794-2597 (International), passcode 3528260. 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 3528260.

About IVERIC bio

IVERIC bio is a science-driven biopharmaceutical company with a focus on the discovery and development of novel gene therapy solutions to treat orphan inherited retinal diseases with unmet medical needs. Vision is Our Mission. For more information on the Company’s gene therapy and other programs, 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,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions. In this press release, the Company’s forward looking statements include statements about the implementation of its strategic plan, including its focus on developing gene therapies for orphan inherited retinal diseases, 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, including its collaborative gene therapy sponsored research programs and any potential in-license or acquisition opportunities. 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 in-license and/or acquisition 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 June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 10,009	$ 8,516	$ 17,694	$ 16,202
General and administrative	5,198	6,332	10,679	11,977
Total operating expenses	15,207	14,848	28,373	28,179
Loss from operations	(15,207)	(14,848)	(28,373)	(28,179)
Interest income	617	602	1,287	1,075
Other income (expense)	151	-	151	(16)
Loss before income tax provision (benefit)	(14,439)	(14,246)	(26,935)	(27,120)
Income tax provision (benefit)	4	(1,037)	9	(838)
Net loss	$ (14,443)	$ (13,209)	$ (26,944)	$ (26,282)
Net loss per common share:				
Basic and diluted	$ (0.35)	$ (0.37)	$ (0.65)	$ (0.73)
Weighted average common shares outstanding:				
Basic and diluted	41,477	36,188	41,452	36,171

	June 30, 2019	**December 31, 2018**
	(in thousands)	
Balance Sheets Data:		
Cash and cash equivalents	$ 106,889	$ 131,201
Total assets	113,608	137,165
Total liabilities	11,875	13,206

Additional paid-in capital	550,303	545,585
Accumulated deficit	(448,611)	(421,667)
Total stockholders' equity	$ 101,733	$ 123,959

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