# Iveric Bio Announces Successful Advancement of LCA10 Minigene Program and Expansion of Orphan Gene Therapy Portfolio with USH2A-Related Inherited Retinal Diseases Program

July 23, 2019

NEW YORK--(BUSINESS WIRE)--Jul. 23, 2019-- IVERIC bio, Inc. (Nasdaq: ISEE) today announced that the Company has exercised its option and entered into an exclusive global license agreement with the University of Massachusetts Medical School for rights to develop and commercialize mutation independent novel adeno-associated virus (AAV) gene therapy product candidates for the treatment of Leber Congenital Amaurosis type 10 (LCA10) due to mutations to the *CEP290* gene, the most common type of LCA. IVERIC bio's collaboration with UMass Medical School and its Horae Gene Therapy Center, utilizing the minigene therapy approach, has resulted in additional research data that supports the Company's plans to move the program forward.

The Company also announced today that it is expanding its gene therapy portfolio by entering into a sponsored research agreement with UMass Medical School and an exclusive option agreement for rights to develop and commercialize novel AAV gene therapy product candidates utilizing a mutation independent minigene therapy approach for the treatment of vision loss in *USH2A*-related inherited retinal diseases (IRDs). This is a group of orphan IRDs that includes Usher syndrome type 2A and *USH2A*-associated autosomal recessive nonsyndromatic retinitis pigmentosa.

"Moving our LCA10 program forward and expanding our pipeline with the addition of a minigene research program for *USH2A* further validates our commitment to develop innovative and life changing gene therapies for patients with orphan inherited retinal diseases," stated Glenn P. Sblendorio, Chief Executive Officer and President of IVERIC bio. "We are very excited about the progress of our collaboration with world-renowned AAV gene therapy scientists at UMass Medical School, Hemant Khanna, PhD, and Guangping Gao, PhD."

"The results from our LCA10 minigene program are encouraging for the prospect of using AAV vectors for the treatment of retinal diseases caused by gene mutations that exceed the transgene packaging capacity of conventional AAV vectors," stated Kourous A. Rezaei, MD, Chief Medical Officer of IVERIC bio. "With the addition of the *USH2A* program to our portfolio, IVERIC bio is seeking to address vision loss associated with what we believe are the most common autosomal dominant form (rhodopsin-mediated) and autosomal recessive form (*USH2A*-related) of retinitis pigmentosa. Retinitis pigmentosa is the most common orphan inherited retinal disease."

IVERIC bio plans to update investors on all of the Company's gene therapy programs at its upcoming Gene Therapy R&D Investor Day scheduled for September 13, 2019.

**About Minigene Therapy**

The use of minigene therapy as a therapeutic strategy seeks to deliver a smaller but functional portion of the larger gene packaged into a standard-size AAV delivery vector commonly used in ocular gene therapy. Research in this evolving area of gene therapy is led by Drs. Hemant Khanna and Guangping Gao in the Horae Gene Therapy Center at UMMS and was described in the 2018 *Human Gene Therapy* journal article, "Gene Therapy Using a miniCEP290 Fragment Delays Photoreceptor Degeneration in a Mouse Model of Leber Congenital Amaurosis" by Wei Zhang, Linjing Li, Qin Su, Guangping Gao, and Hemant Khanna, all of whom are affiliated with the University of Massachusetts Medical School.

**About Leber Congenital Amaurosis Type 10**

Leber Congenital Amaurosis type 10 (LCA10), the most common type of LCA, is an orphan inherited retinal disease caused by mutations in the *CEP290* gene. Mutations in *CEP290* lead to severe bilateral vision loss in early childhood, incapacitating affected individuals.

**About *USH2A*-Related Inherited Retinal Diseases**

*USH2A*-related inherited retinal diseases (IRDs) are a group of orphan IRDs associated with mutations to the *USH2A* gene. The *USH2A* gene encodes a protein, usherin, which is believed to be important in the development and maintenance of cells in the retina and the inner ear. *USH2A*-related IRDs include retinitis pigmentosa associated with Usher syndrome type 2A and *USH2A*-associated autosomal recessive nonsyndromatic retinitis pigmentosa. Usher syndrome type 2a is an autosomal recessive genetic condition characterized by hearing loss from birth and progressive vision loss, due to retinitis pigmentosa, that begins in adolescence or adulthood. *USH2A*-associated autosomal recessive nonsyndromatic retinitis pigmentosa is a genetic condition that manifests as vision loss without associated hearing loss. The collaborative research program between IVERIC bio and UMMS seeks to develop an AAV deliverable, mutation independent, minigene treatment option for the vision loss associated with *USH2A* mutations.

**About IVERIC bio**

IVERIC bio is a biotechnology 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," "prospect," "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, 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 any potential in-license or acquisition opportunities. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company's research and 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 conduct and design of research programs, preclinical studies and clinical trials, availability of data from these activities, 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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