**Nonhuman Primate Data from iECURE’s Knock-in *In Vivo* Gene Editing Approach for Deadly Urea Cycle Disorder to be Presented in Presidential Symposium at ASGCT 2022**

*Mutation-agnostic approach was well tolerated and led to sustained expression of therapeutic gene*

**PHILADELPHIA**—May 18, 2022 - [iECURE](https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fiecure.com%2F&esheet=52571805&newsitemid=20220201005511&lan=en-US&anchor=iECURE&index=1&md5=b42a9bb5996399d03fc8549a2e4fef8d), a gene editing company focused on mutation-agnostic *in vivo* gene insertion, or knock-in, editing for the treatment of liver disorders with significant unmet need, today announced that data showing the potential for the company’s gene editing approach to treating ornithine transcarbamylase (OTC) deficiency, a urea cycle disorder, will be presented in the Presidential Symposium and Presentation of Top Abstracts Plenary Session at the American Society of Gene & Cell Therapy (ASGCT) annual meeting, being held in Washington, D.C.

“The selection of this abstract for the highly regarded Presidential Symposium highlights both the innovation and the potential of our OTC program, and broader pipeline,” said Joseph Truitt, Chief Executive Officer of iECURE. “Importantly, the robust data show unprecedented efficiency of *in vivo* gene targeting and well-tolerated durable expression of the therapeutic gene in both newborn and infant nonhuman primates, or NHPs. We will continue to work closely with our partners at University of Pennsylvania’s Gene Therapy Program to generate the data required to start clinical testing in OTC patients.”

iECURE’s approach to gene editing for its initial programs, including OTC deficiency, citrullinemia type 1 (CTLN1) and phenylketonuria (PKU), relies on the delivery of twin adeno-associated virus (AAV) capsids carrying different payloads: one with a gene encoding for the ARCUS® nuclease to cut genomic DNA at the well-characterized PCSK9 locus and the other with a therapeutic donor gene.[[1]](#footnote-1) The cut in the PCSK9 site serves as the insertion site for the therapeutic gene, providing a potential path to permanent expression of a healthy gene.

The data, to be presented by Lili Wang, Ph.D., Research Director, Translational Research and Gene Editing, Research Associate Professor, Department of Medicine at the University of Pennsylvania Gene Therapy Program, focus on the insertion of two different therapeutic genes, one coding for factor IX and one coding for OTC. Long-term gene expression data was generated using the gene that encodes human factor IX (hFIX) in NHPs, with evidence showing 30-60% of normal levels of hFIX expression in newborn and three-month olds for 17 to 20 months. Gene editing was remarkably well tolerated based on weight gain and alanine transaminase (ALT) levels.

Using the same dual vector construct, but with an OTC donor gene, researchers demonstrated highly efficient and well-tolerated *in vivo* insertion in newborn NHPs based on the 3-month biopsy.

“One of the greatest challenges for using healthy genes to treat liver disorders affecting infants is the rapid division of liver cells, which prevents long-term expression of genes that have not integrated into the genome,” said James M. Wilson, M.D., Ph.D., Rose H. Weiss professor and director, Orphan Disease Center; professor in the Departments of Medicine and Pediatrics, Perelman School of Medicine; and director of the Gene Therapy Program (GTP) at the University of Pennsylvania. “The data we generated show exceptional promise for long-term expression of therapeutic genes, which we believe could help address the unmet medical needs for children with a range of inborn errors of metabolism, many of which result in death at a far too early age.”

The presentation, entitled “AAV-Meganuclease-Mediated Gene Targeting Achieves Efficient and Sustained Transduction in Newborn and Infant Macaque Liver,” will be presented on May 18, 2022, from 3:00 - 3:15 PM in Hall E at the Walter E. Washington Convention Center.

**About iECURE**iECURE is a gene editing company focused on developing therapies that utilize mutation-agnostic *in vivo* gene insertion, or knock-in, editing for the treatment of monogenic liver disorders with significant unmet need. We believe our approach has the potential to replace and restore the function of a dysfunctional gene by knocking-in a healthy copy, regardless of mutation, to offer durable gene expression and long-term, potentially curative, therapeutic benefit. Our management team has extensive experience in executing global orphan drug and gene therapy clinical trials and successfully commercializing multiple products. We intend to leverage our team’s core strength in research and development strategy to identify what we believe to be the most suitable target and modality for our product candidates to address particular liver diseases. We are collaborating with the University of Pennsylvania’s Gene Therapy Program, or GTP, led by James M. Wilson, M.D., Ph.D., to utilize GTP’s world-class translational expertise and infrastructure, which has helped generate our initial pipeline of potential product candidates. For more information, visit [***www.iecure.com***](https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fiecure.com%2F&esheet=52571805&newsitemid=20220201005511&lan=en-US&anchor=www.iecure.com&index=2&md5=239987cdc9bfa39fad3a818d8e48a9d8) and follow on [***LinkedIn***](https://cts.businesswire.com/ct/CT?id=smartlink&url=https%3A%2F%2Fwww.linkedin.com%2Fcompany%2Fiecure%2F&esheet=52571805&newsitemid=20220201005511&lan=en-US&anchor=LinkedIn&index=3&md5=32106a1d6f7e2c86dfeaeb2cbc3e77d3).

Financial disclosure: The University of Pennsylvania (Penn) and Dr. Wilson hold equity interests in iECURE. Penn also receives significant sponsored research support from the company, and both Penn and Dr. Wilson benefit from licensing revenues received from iECURE based on successful technology development and commercialization of the technologies licensed from Penn. Dr. Wilson serves as Chief Scientific Advisor for iECURE.

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1. iECURE has licensed the ARCUS® nuclease from Precision BioSciences for four gene insertion programs including OTC, CTLN1 and PKU [↑](#footnote-ref-1)