iECURE Receives FDA Rare Pediatric Disease Designation for GTP-506, an Investigational Gene Editing Product Candidate for the Treatment of Ornithine Transcarbamylase (OTC) Deficiency

- *FDA decision recognizes OTC deficiency as a "rare disease or condition"*
- *GTP-506, a potential single-dose gene editing therapy designed to restore metabolic function in patients suffering with OTC deficiency, a rare urea cycle disorder*
- *iECURE on-track to submit an Investigational New Drug (IND) application for GTP-506 in mid-2023*

PHILADELPHIA—August 23, 2022 - **iECURE**, a gene editing company focused on developing therapies that utilize mutation-agnostic *in vivo* gene insertion, or knock-in, editing for the treatment of liver disorders with significant unmet need today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to its lead product candidate GTP-506 for the treatment of Ornithine Transcarbamylase (OTC) deficiency, a rare genetic condition that can lead to irreversible neurological impairment, seizures, coma and death, in a pediatric population.

"Receiving Rare Pediatric Disease Designation for GTP-506 for the treatment of OTC deficiency highlights the dire need for new treatment options for this devastating pediatric disease," said Joe Truitt, Chief Executive Officer of iECURE. "GTP-506 is a potentially transformative therapy for babies born with OTC deficiency, and we expect to file an IND application with the FDA for our first-in-human clinical trial in mid-2023."

The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. The Rare Pediatric Disease Priority Review Voucher Program is intended to address the challenges that drug companies face when developing treatments for these unique patient populations. Under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may be eligible for a voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product or sold to another sponsor for priority review of their marketing application.

About GTP-506

iECURE's approach to gene editing for its initial programs, including OTC deficiency, relies on the delivery of twin adeno-associated virus (AAV) capsids carrying different payloads. GTP-506 comprises two vectors, an ARCUS® nuclease vector (GTP-506A) targeting gene editing in the well-characterized PCSK9 gene locus and a therapeutic donor vector (GTP-506D) that inserts the OTC gene to provide the desired genetic correction.[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.

About OTC Deficiency

[1] iECURE has licensed the ARCUS® nuclease from Precision BioSciences for four gene insertion programs including OTC, CTLN1 and PKU.

OTC deficiency, the most common urea cycle disorder, is an inherited metabolic disorder caused by a genetic defect in a liver enzyme responsible for detoxification of ammonia. Individuals with OTC deficiency can build-up excessive levels of ammonia in their blood, potentially resulting in devastating consequences, including cumulative and irreversible neurological damage, coma and death. The severe form of the condition emerges shortly after birth and is more common in boys than girls. The only treatment for early onset severe OTC deficiency is a liver transplant. Currently available medical therapies do not correct the disease, and do not eliminate the risk of life-threatening symptoms or crises.

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 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*** and follow on ***LinkedIn***.

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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