European Commission Grants Orphan Designation for iECURE's Lead Product Candidate GTP-506 for the Treatment of Ornithine Transcarbamylase (OTC) Deficiency

PHILADELPHIA—March 22, 2023 - **iECURE**, a gene editing company focused on the development of mutation-agnostic *in vivo* gene insertion, or knock-in, editing therapies for the treatment of liver disorders with significant unmet need, announced today that the European Commission granted orphan designation for the company's lead product candidate GTP-506 for the treatment of Ornithine Transcarbamylase (OTC) deficiency.

GTP-506 previously received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) for the treatment of OTC deficiency.

"Securing Orphan Drug designation in both the U.S. and the EU highlights the work we have done to demonstrate how our *in vivo* gene editing approach has the potential to provide clinical benefit to patients who are in desperate need for treatments for OTC deficiency," said Joe Truitt, Chief Executive Officer of iECURE. "Without programs like Orphan designation, thousands of patients with a myriad of rare diseases would have no medicines to treat their disorders, leading to uncontrolled symptoms and possible death. Achieving this designation brings us one step closer to providing a potentially transformative therapy to children living with this devastating disease."

Orphan designation in the European Union (EU) is granted by the European Commission based on a positive opinion issued by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products. The European Commission grants orphan status for products intended for the treatment, prevention, or diagnosis of life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in the EU, and where there is no satisfactory method of treating, preventing, or diagnosing such condition authorized for marketing in the EU or if such a method exists, the product represents a significant benefit to those affected by the condition. Orphan designation provides companies with certain benefits and incentives in the EU, including clinical protocol assistance, possible waivers or reductions in regulatory fees and ten years of market exclusivity after approval.

About OTC Deficiency

OTC deficiency, the most common urea cycle disorder, is an inherited metabolic disorder caused by a genetic defect in a liver enzyme responsible for the detoxification of ammonia. Individuals with OTC deficiency can build-up excessive levels of ammonia in their blood potentially resulting in devastating consequences, including 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 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. iECURE has licensed the ARCUS nuclease for GTP-506 from Precision BioSciences.[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 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 (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***.

About Precision BioSciences & ARCUS
Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (Nasdaq: DTIL) with its novel and proprietary ARCUS® genome editing platform. ARCUS is a highly precise and versatile genome editing platform that was designed with therapeutic safety, delivery, and control in mind. Using ARCUS, the Company's pipeline consists of multiple ex vivo "off-the-shelf" CAR T immunotherapy clinical candidates and several in vivo gene editing candidates designed to cure genetic and infectious diseases where no adequate treatments exist. For more information about Precision BioSciences, please visit **www.precisionbiosciences.com**.

Penn's Financial Disclosure
The University of Pennsylvania (Penn) and Dr. Wilson each hold equity interests in iECURE. Penn also receives significant sponsored research support from the Company, and both Penn and Dr. Wilson stand to 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.