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iECURE Launches With $50 Million Series A Financing to Develop In Vivo Gene Insertion Approaches for Devastating Diseases

- Financing led by Versant Ventures and OrbiMed Advisors -
- Management team composed of seasoned industry executives with proven track records including CEO Joseph Truitt, COO Paul Firuta and CBO Alex Monteith -

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PHILADELPHIA--(BUSINESS WIRE)--iECURE (pronounced EE-ah-cure), a mutation-agnostic in vivo gene editing company striving to cure devastating diseases with high unmet need, today announced it has closed a $50 million Series A financing led by Versant Ventures and OrbiMed Advisors. The funds will be used to advance iECURE's pipeline of up to 13 programs, all of which benefit from gene editing and in vivo delivery approaches being developed in the laboratory of James Wilson, M.D., Ph.D., of the University of Pennsylvania Gene Therapy Program (GTP).

iECURE's approach focuses on the mutation-agnostic in vivo insertion of healthy copies of disease-causing genes, with an initial focus in liver disorders. This groundbreaking strategy has the potential to provide durable benefits for a variety of diseases, particularly in dividing tissues such as the livers of young children. In addition, since a complete gene is inserted into the patient's chromosome, the treatment approach is independent of the specific disease-causing mutation. In primate models, iECURE's programs have shown the ability to integrate wild-type genes into the genome, leading to long-term expression.

"Through our foundational collaboration with Penn, we are gaining access to a comprehensive, liver-focused, in vivo gene editing program that Dr. Wilson and his team have been developing in his labs for several years," said Joseph Truitt, chief executive officer of iECURE. "We are excited to partner with Penn to develop potentially groundbreaking treatments for patients suffering from debilitating disorders of the liver."

A highly enabled pipeline

The Penn collaboration provides iECURE with exclusive licensing rights to three liver disorder programs under current study at GTP and the option to license over 10 additional pipeline assets in the future. Under the joint research and development initiative, GTP will continue to lead preclinical research activities in collaboration with iECURE until company sponsored Investigational New Drug (IND) Application submissions have been filed. Post-IND, iECURE will execute the clinical studies and prepare for potential commercialization.

"We are excited to work with iECURE to further the development of genome editing for metabolic liver disorders which we believe may be successful in disorders where prior gene therapy approaches have fallen short, such as severe neonatal onset liver disorders," said Dr. Wilson, 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. “This partnership further leverages our deep understanding of these diseases and our 34-year experience in gene delivery and translational research.”

iECURE’s initial developmental programs rely on the use of an endonuclease-based gene editing technology delivered via AAV. Dr. Wilson’s laboratory has been studying this approach in several model systems to be further developed via the partnership with iECURE and has generated initial data that supports this approach.

Subsequent programs may incorporate additional technologies for editing payload delivery, and iECURE may pursue in-license of additional technologies to deliver genetic material to liver cells.

A seasoned leadership team

iECURE’s management team members have deep biotechnology experience in rare diseases, gene therapy and in disorders impacting the liver. In addition, they have proven track records of successful exits.

- Mr. Truitt previously was CEO of BioSpecifics Technologies Corp., which was recently acquired by Endo Pharmaceuticals. Prior to BioSpecifics, Mr. Truitt was CEO of Achillion Pharmaceuticals, which he joined in 2009 and held positions of increasing responsibility throughout his tenure. Mr. Truitt is Chairman of the board at Larimar Therapeutics and serves on the board of Code BioTherapeutics.
- iECURE chief operating officer Paul Firuta has nearly 30 years of commercial and operational experience in the biopharma industry, most recently serving as COO of Achillion. Prior, he held the role of chief commercial officer at uniQure, as well as other senior commercial leadership positions at NPS Pharmaceuticals and ViroPharma.
- Alex Monteith, iECURE chief business officer, has over two decades of experience in the biopharmaceutical space. His most recent position was at BioSpecifics Technologies, where he served as CBO with Mr. Truitt. Prior to BioSpecifics, he was VP of business development for Deerfield Management. He has also served as VP of business development for Achillion.

iECURE’s board of directors includes Mr. Truitt; Stephen Squinto, Ph.D., venture partner at OrbiMed Advisors; and Chairman Tom Woiwode, Ph.D., managing director at Versant Ventures.

“Versant has made several important investments in the gene editing space, including founding Crispr Therapeutics and Graphite Therapeutics, and we believe that iECURE represents the next wave of innovation in this space,” said Dr. Woiwode. “We’re thrilled to be working with Jim and his team, as well as with our colleagues at OrbiMed and the founding team at iECURE, to push forward these breakthrough therapeutic approaches for patients suffering from liver disorders.”

Financial disclosure: The University of Pennsylvania and Dr. Wilson hold equity interests in iECURE, receive significant sponsored research support from the company, and will be entitled to receive licensing revenues from iECURE based on successful technology development and commercialization of the technologies licensed from Penn.

About iECURE

iECURE is a mutation-agnostic in vivo gene editing company striving to cure devastating diseases with high unmet need. We are advancing our pipeline in close partnership with the world-class translational engine at the University of Pennsylvania’s Gene Therapy Program. Using in vivo editing, our methods focus on inserting functioning genes into

patients' genomes, which offers long-term, stable expression of those genes. With our team's proven track record, as well as the University of Pennsylvania's deep expertise and translational genetic medicine engine, reversing the course of these devastating diseases is now within reach.

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