

# IECURE PARTNERS TO DEVELOP NEXT-GENERATION LIVER-TARGETED LIPID NANOPARTICLES WITH UNIVERSITY OF PENNSYLVANIA

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**PHILADELPHIA—(BUSINESS WIRE)**—iECURE, a mutation-agnostic *in vivo* gene editing company striving to cure devastating diseases with significant unmet need, today announced that it has entered into an exclusive agreement with the University of Pennsylvania (Penn) to develop next-generation lipid nanoparticles (LNP) for liver gene editing applications. This LNP technology comes out of the laboratory of Michael Mitchell, Ph.D.

“Our focus on gene insertion approaches to correct liver disorders relies on the most robust delivery of gene editing enzymes for the application at hand,” said Joseph Truitt, chief executive officer of iECURE. “Having the adaptability to go beyond AAV to deliver the gene editing payload is important, particularly as we explore adult indications or if the use of an alternative gene editing technology delivers stronger data. Given that, this collaboration with Dr. Mitchell’s lab is critical as we lay out our strategic path for liver-focused gene editing-based therapies.”

iECURE will have certain exclusive rights to gene editing in the liver with LNP technology developed in collaboration with Dr. Mitchell. In addition, the agreement expands the number of potential pipeline programs to be developed by iECURE with Penn from 13 to 15.

Dr. Mitchell is the Skirkanich Assistant Professor of Innovation in the Department of Bioengineering at the University of Pennsylvania School of Engineering and Applied Science. Dr. Mitchell’s laboratory is a world leader in the discovery and development of biomaterials for overcoming biological barriers to drug delivery, with a particular focus on the development of next-generation LNPs for the delivery of mRNA, siRNA, and gene editing therapeutics. The laboratory has collaborated for multiple years with Penn’s Gene Therapy Program (GTP), which is directed by James M. Wilson, M.D., Ph.D.; this

collaboration will continue under the Penn-iECURE partnership. Prior to joining Penn in 2018, Dr. Mitchell was a National Institutes of Health (NIH) Postdoctoral Fellow in the laboratory of Robert S. Langer, Sc.D., of the Massachusetts Institute of Technology.

“As gene editing moves to the forefront for correcting genetic diseases, scientists will discover new editing technologies that may require alternative means of delivery,” noted Dr. Mitchell. “Our laboratory has worked to develop non-viral vectors for nucleic acid delivery, and we are finding that LNP technologies work very well for delivering a range of gene editing technologies. We are pleased to be collaborating with the team at iECURE.”

iECURE’s initial internal developmental programs rely on the use of endonuclease-based gene editing technology delivered via AAV for rare neonatal indications. Subsequent pipeline candidates may use LNPs for delivery of other gene editing technologies.

Financial disclosure: The University of Pennsylvania and Dr. Wilson hold equity interests in iECURE. The University of Pennsylvania, Dr. Wilson and Dr. Mitchell receive 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 licensed technology. Dr. Mitchell is a scientific advisor for iECURE.

## ABOUT IECURE

iECURE is a mutation-agnostic *in vivo* gene editing company striving to cure devastating diseases with significant 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 functional genes into patients’ genomes, offering 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, we believe reversing the course of these devastating diseases is now within reach. For more information, visit [www.iecure.com](http://www.iecure.com) and follow on [LinkedIn](#).

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