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Penn spinout iEcure raises $65M to advance its gene-editing technology

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Joseph Truitt, CEO of iEcure.

A University of Pennsylvania spinout has raised $65 million to advance its in vivo gene-editing technology aimed at treating rare pediatric liver diseases.

The Series A-1 equity financing round for iEcure was co-led by Novo Holdings and Lyfe Capital. Existing investors Versant Ventures and OrbiMed Advisors also participated in the round.

IEcure, which is based in Plymouth Meeting but is in the process of moving into its new headquarters in Blue Bell, previously raised $50 million in a Series A financing that closed in September 2021.

The company plans to use the new funding to complete pre-clinical work, initiate clinical trials and generate clinical data from a Phase 1/2 study of its lead investigational product for ornithine transcarbamylase deficiency. Ornithine transcarbamylase

deficiency is a rare condition caused by a genetic defect in a liver enzyme responsible for the detoxification of ammonia.

IEcure is targeting the early occurrence of the disorder — which can lead to irreversible neurological impairment, seizures, coma, and death. An early onset form of the disorder is found in about 100 births, most commonly in newborn boys, annually. According to the company, the only treatment currently available for the severe forms of the condition is a liver transplant. Available medical therapies, iEcure noted, do not correct the disease — and do not eliminate the risk of life-threatening symptoms.

Joseph Truitt, iEcure's CEO, said during the last year the company has made "significant progress" in both advancing its lead drug candidate and building its team of experienced life sciences executives and researchers with a track record in developing and commercializing novel therapies.

"We are at 12 people now and we expect to add another dozen [over the next year]," Truitt said.

Truitt said the company is working with two contract manufacturers — Catalent, a global company with offices in Philadelphia and Malvern, and the Center for Breakthrough Medicines in King of Prussia — rather than attempting to do manufacturing in-house, which would require an additional 80 to 100 employees.

The gene-editing approach being taken by iEcure — which is collaborating with the University of Pennsylvania's Gene Therapy Program — involves making a cut in a patient's DNA and inserting a healthy copy of the disease-causing gene into the chromosome.

"We believe that this funding will enable us to execute all the tasks necessary to begin clinical development of what could be the first mutation-agnostic in vivo gene insertion therapeutic program," Truitt said.

The company's long-term plans are to expand the use of the treatment to all patients with ornithine transcarbamylase deficiency.

"We think in gene-editing it's best to walk before you run," Truitt said.

IEcure also plans to use a portion of the funds for preclinical testing of another new drug candidate targeting citrullinemia type 1, a rare autosomal recessive genetic disorder in which ammonia and other toxic substances build up in the blood and cause life-threatening complications shortly after birth.

As part of the Series A-1 financing, Ray Camahort, a partner at Novo Ventures, and Derek Yuan, managing director at Lyfe Capital, will join iEcure's board of directors. In addition, Dr. Tal Zaks was named to iEcure's board as Orbimed's new representative on the board. Steve Squinto, one of the company's co-founders, will continue to serve on the board as an independent member.

In addition to Squinto, iEcure's other founders are Dr. Jim Wilson, director of Penn's gene therapy program, and the late Dr. Tadataka "Tachi" Yamada, a former executive at Takeda Pharmaceuticals and GlaxoSmithKline.

Truitt acknowledged this is a difficult climate for life sciences companies to raise money from private and public investors, who are looking for safer places to invest during a down economy. Securing new investment capital has been particularly tough, he said, for early-stage life sciences companies "too far from the clinic" and those companies that went public too early and have valuation issues.

He believes iEcure benefitted from the strong preclinical data generated by Wilson and Penn's gene therapy program, and the experienced management team the company has assembled to advance the technology.

"And there's tremendous interest in the gene-editing space," Truitt said.

John George
Senior Reporter - *Philadelphia Business Journal*

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