



Precision BioSciences and iECURE Announce License and Collaboration Agreement to Develop ARCUS-Based Gene Editing Therapies

September 9, 2021 at 7:01 AM EDT

iECURE to License Precision's PCSK9-Directed ARCUS Nuclease and Pursue Four Gene Insertion Programs Focused on Liver Diseases; Precision Retains Rights Across All Indications Except Those Licensed to iECURE

iECURE Expects to File a Clinical Trial Application as Early as 2022 for Precision's PBGENE-PCSK9 Candidate for Familial Hypercholesterolemia

Preclinical Data for ARCUS-Mediated Gene Insertion to be Presented Today by iECURE Chief Scientific Advisor, James M. Wilson, M.D., Ph.D. at Precision's Gene Editing R&D Event

DURHAM, N.C. & PHILADELPHIA--(BUSINESS WIRE)--Sep. 9, 2021-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical-stage biotechnology company developing allogeneic CAR T and *in vivo* gene correction therapies with its ARCUS[®] genome editing platform, and iECURE, a mutation-agnostic *in vivo* gene editing company striving to cure devastating diseases with high unmet need, today announced a license and collaboration agreement under which iECURE plans to advance Precision's PBGENE-PCSK9 candidate into Phase 1 studies and gain access to Precision's PCSK9-directed ARCUS nuclease to develop additional gene editing therapies for genetic diseases, initially targeting liver diseases.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20210909005309/en/>

Under the terms of the agreement, iECURE plans to file a clinical trial application as early as 2022 to advance the PBGENE-PCSK9 clinical candidate through Phase 1 clinical studies for the treatment of familial hypercholesterolemia (FH). Precision will retain rights to PBGENE-PCSK9, including all products developed for genetic indications with increased risk of severe cardiovascular events such as FH. In return, Precision has granted iECURE a license to use its PCSK9-directed ARCUS nuclease to insert genes into the well-characterized PCSK9 locus to develop treatments for four other pre-specified rare genetic diseases. Precision will receive an equity stake in iECURE and is eligible to receive milestone and royalty payments on sales of iECURE products developed with ARCUS.

"We are excited to continue working with Jim Wilson under this new *in vivo* gene editing license and collaboration agreement with iECURE, as iECURE looks to rapidly advance our PBGENE-PCSK9 candidate, file for a clinical trial application in 2022, and use our PCSK9-directed ARCUS nuclease, and its knock-in capabilities, to pursue new treatments for rare genetic diseases," said Derek Jantz, Ph.D., Chief Scientific Officer and Co-Founder of Precision BioSciences. "Through this collaboration we expect to gain important clinical validation for *in vivo* gene editing with ARCUS, while retaining rights to this PCSK9-directed nuclease, which we believe offers a safe harbor locus for DNA gene editing knock-in without deleterious effects when the PCSK9 gene is disrupted."

"We founded iECURE with the aim of focusing on genetic diseases with significant unmet need that we could target in a mutation-agnostic manner. After evaluating different gene editing technologies and platforms, we believe gene editing with ARCUS, including use of the uniquely designed ARCUS nuclease as a gene insertion tool targeting the PCSK9 gene will help us rapidly advance several candidates to the clinic with the potential to deliver on the promise of highly efficient, specific, and safe gene insertion," said Joe Truitt, Chief Executive Officer of iECURE. "We are excited to partner with Precision on this key pillar of our gene editing strategy, to advance this work for rare genetic diseases."

James M. Wilson, M.D., Ph.D., Chief Scientific Advisor of iECURE and Professor in the Departments of Medicine and Pediatrics, Perelman School of Medicine, University of Pennsylvania, Director, Gene Therapy Program, will present new non-human primate data demonstrating ARCUS-mediated gene addition today, September 9, 2021 during the [Precision BioSciences gene editing R&D event](#). Dr. Wilson and his team have demonstrated in non-human primates that it is possible to use ARCUS to insert new genes stably into the PCSK9 locus, which could be used as a potential approach for treating multiple genetic diseases with a single therapeutic strategy.

About ARCUS

ARCUS[®] is a proprietary genome editing technology discovered and developed by scientists at Precision BioSciences. It uses sequence-specific DNA-cutting enzymes, or nucleases, that are designed to either insert (knock in), remove (knockout), or repair DNA of living cells and organisms. ARCUS is based on a naturally occurring genome editing enzyme, I-CreI, that evolved in the algae *Chlamydomonas reinhardtii* to make highly specific cuts in cellular DNA. Precision's platform and products are protected by a comprehensive portfolio including more than 80 patents to date.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its novel and proprietary ARCUS[®] genome editing platform. ARCUS is a highly specific 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 "off-the-shelf" CAR T immunotherapy clinical candidates and several *in vivo* gene correction therapy candidates to cure genetic and infectious diseases where no adequate treatments exist. For more information about Precision BioSciences please visit www.precisionbiosciences.com.

About iECURE

iECURE is an *in vivo* gene editing company striving to cure liver disorders 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, reversing the course of these devastating disorders is now hopefully within reach.

Financial disclosure: The University of Pennsylvania and Dr. James 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 licensed technology.

Precision's Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the Company's agreement with iECURE to develop and commercialize gene editing therapies using the Company's PCSK9-directed ARCUS nuclease for cardiovascular and rare genetic diseases, the timing of clinical trials and results therefrom, any future milestones or royalty payments thereunder, the development and commercial potential of ARCUS-mediated gene addition and the potential value of iECURE equity. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "believe," "could," "expect," "should," "plan," "intend," "estimate," "target," "mission," "goal," "may," "will," "would," "should," "could," "target," "potential," "project," "predict," "contemplate," "potential," or the negative thereof and similar words and expressions.

Forward-looking statements are based on management's current expectations, beliefs and assumptions and on information currently available to us. Such statements are subject to a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to: our ability to become profitable; our ability to procure sufficient funding and requirements under our current debt instruments and effects of restrictions thereunder; risks associated with raising additional capital; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the success of our programs and product candidates in which we expend our resources; our limited ability or inability to assess the safety and efficacy of our product candidates; our dependence on our ARCUS technology; the initiation, cost, timing, progress, achievement of milestones and results of research and development activities, preclinical or greenhouse studies and clinical or field trials; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, biotechnology and agricultural biotechnology fields; our or our collaborators' ability to identify, develop and commercialize product candidates; pending and potential liability lawsuits and penalties against us or our collaborators related to our technology and our product candidates; the U.S. and foreign regulatory landscape applicable to our and our collaborators' development of product candidates; our or our collaborators' ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate; our or our collaborators' ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; our ability to obtain an adequate supply of T cells from qualified donors; our ability to achieve our anticipated operating efficiencies at our manufacturing facility; delays or difficulties in our and our collaborators' ability to enroll patients; changes in interim "top line" and initial data that we announce or publish; if our product candidates do not work as intended or cause undesirable side effects; risks associated with applicable healthcare, data protection, privacy and security regulations and our compliance therewith; the rate and degree of market acceptance of any of our product candidates; the success of our existing collaboration agreements, and our ability to enter into new collaboration arrangements; our current and future relationships with and reliance on third parties including suppliers and manufacturers; our ability to obtain and maintain intellectual property protection for our technology and any of our product candidates; potential litigation relating to infringement or misappropriation of intellectual property rights; our ability to effectively manage the growth of our operations; our ability to attract, retain, and motivate key executives and personnel; market and economic conditions; effects of system failures and security breaches; effects of natural and manmade disasters, public health emergencies and other natural catastrophic events effects of the outbreak of COVID-19, or any pandemic, epidemic or outbreak of an infectious disease; insurance expenses and exposure to uninsured liabilities; effects of tax rules; risks related to ownership of our common stock; and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2021, as any such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC's website at www.sec.gov and the Investors & Media page of our website at investor.precisionbiosciences.com.

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.



View source version on [businesswire.com](https://www.businesswire.com/news/home/20210909005309/en/): <https://www.businesswire.com/news/home/20210909005309/en/>

Precision BioSciences Investor Contact:

Alex Kelly
Chief Financial Officer
Alex.Kelly@precisionbiosciences.com

Precision BioSciences Media Contact:

Maurissa Messier
Senior Director, Corporate Communications
Maurissa.Messier@precisionbiosciences.com

iECURE Contact:

Danielle Cantey
Canale Communications for iECURE
Danielle.cantey@canalecomm.com

Source: Precision BioSciences, Inc.