



Precision BioSciences Announces Paper Presentation at the Association for Research in Vision and Ophthalmology 2021 Annual Meeting

May 3, 2021 at 7:00 AM EDT

Preclinical Research with ARCUS Genome Editing Rejuvenated Rod Photoreceptor Structure and Function in a Transgenic Pig Model of Human Blindness

DURHAM, N.C.--(BUSINESS WIRE)--May 3, 2021-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage biotechnology company developing allogeneic CAR T and *in vivo* gene correction therapies, announced today that the following paper presentation, highlighting preclinical research using its ARCUS® genome editing platform for autosomal dominant Retinitis Pigmentosa (adRP), will be presented today at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting.

Title: Rho 1-2 meganuclease, an allele-specific gene-editing therapy, rejuvenates rod photoreceptor structure and function in a pig model of autosomal dominant Retinitis Pigmentosa (adRP)

Paper Presentation: Gene Therapy - Physiology/Pharmacology, Abstract 3543926

Date/Time: Monday, May 3, 2021, 4:30 – 6:00 p.m. EDT

Presenting Author: Archana Jalligampala, Ph.D., Postdoctoral Associate, Department of Ophthalmology and Visual Sciences, University of Louisville

Co-Authors: Jennifer Noel¹, James W. Fransen¹, Wei Wang¹, Maha H. Jabbar¹, Nazarul Hasan¹, Gobinda Pangeni¹, Bhubanananda Sahu¹, Whitney Lewis², Jeff Smith², Victor Bartsevich², Kristi Viles², Derek Jantz², Maureen A. McCall¹

The P23H mutation in the rhodopsin gene represents the most common form of adRP in North Americans. Mutations in the rhodopsin gene account for 25 to 30 percent of all cases of adRP in the United States. This leads to the progressive loss of rods, which are responsible for vision at low light levels.

"This form of adRP, an inherited eye disease that leads to complete loss of central vision, is not amenable to conventional gene replacement therapies," said Archana Jalligampala, Ph.D., Postdoctoral Associate, Department of Ophthalmology and Visual Sciences, University of Louisville. "We used ARCUS nucleases to target the P23H human rhodopsin mutation and found that, at our latest scheduled structural and functional assessment at 44 weeks post-injection, rods in the treated P23H retinas were more numerous, had elongated outer segments, and correctly localized rhodopsin compared to the untreated area in the same P23H retina or to an untreated P23H retina. We're very encouraged by these results as they indicate that the gene editing approach using ARCUS has the potential to treat adRP in human patients with the same point mutation and, importantly, at late disease stages."

In this study, ARCUS nuclease-treated animals showed significant improvements in rod driven signals, namely an increase in electroretinography (ERG) b-wave response compared to untreated animals. This effect was observed as early as postnatal day (P) 60 and was maintained through P300. These results suggest that the level of gene editing achieved in this study may facilitate functional visual improvements.

"We are excited to see that this latest large animal, preclinical dataset using ARCUS to address adRP will be presented at the annual ARVO meeting," commented Derek Jantz, Ph.D., Co-Founder and Chief Scientific Officer at Precision BioSciences. "This work adds to the growing body of preclinical research where *in vivo* gene editing with ARCUS has resulted in sustained effective responses. We look forward to the continued development of ARCUS for rare genetic conditions like adRP that lack a safe and effective treatment option."

Video-recorded presentations from the ARVO 2021 meeting are available on the [Pathable online platform](#).

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 (knock-out), 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 75 patents to date.

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 potential results, uses and advancement of our *in vivo* gene editing programs and ARCUS-based gene editing technology, including, without limitation, its attributes and effects upon autosomal dominant Retinitis Pigmentosa (adRP). In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "believe," "could," "eligible," "expect," "should," "plan," "intend," "estimate," "target," "mission," "goal," "may," "suggest," "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 Annual Report on Form 10-K for the year ended December 31, 2020, 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.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its wholly 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.

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