



## New Preclinical Data Presented at the 2021 American Society of Genetic & Cell Therapy Annual Meeting Highlights Precision BioSciences' ARCUS In Vivo Genome Editing

May 11, 2021

*Results Show Promising Gene Editing Approach for Transthyretin Amyloidosis*

DURHAM, N.C.--(BUSINESS WIRE)--May 11, 2021-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage biotechnology company developing allogeneic CAR T and *in vivo* gene correction therapies with its ARCUS<sup>®</sup> genome editing platform, announced new *in vivo* data resulting from a preclinical research collaboration with investigators at the University of Pennsylvania presented today at the 2021 American Society of Genetic & Cell Therapy (ASGCT) Annual Meeting.

In the poster, "Translation of an AAV-delivered gene editing approach for transthyretin amyloidosis from mice to nonhuman primates," researchers report positive preclinical results using ARCUS to knock out the transthyretin (TTR) gene responsible for transthyretin amyloidosis (ATTR), a rare disease that leads to peripheral neuropathy and/or cardiomyopathy.

"While there are approved treatment approaches for ATTR, these require repeated administration, whereas a gene editing approach delivered by adeno-associated viral vectors, including the one we're studying with the ARCUS nucleus, could provide an effective one-time treatment option," said Jenny Greig, Ph.D., Senior Director, Gene Therapy Program, Perelman School of Medicine, University of Pennsylvania. "The significant reduction in serum TTR levels following genomic editing of the TTR gene indicates that AAV delivery of TTR-specific ARCUS nucleases could represent an effective treatment for ATTR."

In ARCUS-treated NHPs, high levels of genomic editing were achieved, resulting in substantial and persistent serum TTR reductions. Up to 46% indels were found at the genomic DNA locus, translating to ~80% editing of the TTR messenger RNA transcripts, which was maintained between liver biopsies collected at 18 and 128 days post-AAV administration. Researchers found that high editing efficacy of the TTR gene was reflected in serum TTR levels, where there was an associated reduction of greater than 95% by day 21 post-vector administration in three out of the four ARCUS-treated NHPs. These serum TTR reductions endured to 250 days after a single AAV administration, suggesting the genomic edits and subsequent protein reduction may be permanent.

"Using ARCUS to knock out the TTR gene in NHPs provided an important opportunity to evaluate the persistent effects of ARCUS for *in vivo* editing," said Derek Jantz, Ph.D., Chief Scientific Officer and co-founder of Precision BioSciences. "We're excited about the growing body of data demonstrating this 'one and done' approach with ARCUS, and what it may mean for transforming the treatment of rare genetic diseases in humans."

All abstracts for the ASGCT 2021 Meeting are available on the meeting [website](#).

### About ARCUS

ARCUS<sup>®</sup> 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.

### About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its wholly proprietary ARCUS<sup>®</sup> 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](http://www.precisionbiosciences.com).

### 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 transthyretin amyloidosis (ATTR). 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](http://www.sec.gov) and the Investors & Media page of our website at [investor.precisionbiosciences.com](http://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.

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