



Precision BioSciences Presents Preclinical Data Highlighting Capabilities of ARCUS for Gene Insertion and Excision at the European Society of Gene & Cell Therapy (ESGCT) 30th Annual Congress

October 26, 2023 at 7:30 AM EDT

- ARCUS showed durable, high-efficiency gene insertion capabilities in NHPs and comparison to CRISPR-Cas9

- Data demonstrates ability of ARCUS to achieve large gene excisions enabling significant functional muscle improvement in a DMD mouse model

DURHAM, N.C.--(BUSINESS WIRE)--Oct. 26, 2023-- Precision BioSciences, Inc. (Nasdaq: DTIL), an advanced gene editing company utilizing its novel proprietary ARCUS® platform to develop *in vivo* gene editing therapies for sophisticated gene edits, including gene insertion, excision, and elimination, today announced that the company will present two posters at the ESGCT 30th Annual Congress that highlight ARCUS' differentiated ability to make efficient, durable, and targeted insertion edits in non-human primates (NHPs), as well as preclinical data demonstrating the potential of ARCUS to achieve large excision of a commonly mutated region of the dystrophin gene for the potential treatment of Duchenne muscular dystrophy (DMD).

"We believe ARCUS has the potential to redefine gene editing by enabling sophisticated gene edits such as gene insertion, excision, and elimination," said Jeff Smith, Chief Research Officer of Precision BioSciences. "ARCUS' ability to create 4 base pair, 3' overhangs is designed to make predictable, highly consistent, therapeutic edits and enable high efficiency gene insertion through homology directed repair (HDR). In preclinical work presented today at ESGCT, ARCUS demonstrated 17 times higher gene insertion efficiency compared to CRISPR-Cas9, validating the importance of the cut made by ARCUS. In addition, data presented at ESGCT continue to support ongoing development of our gene excision program focused on DMD, in development with Prevail Therapeutics, a wholly-owned subsidiary of Eli Lilly and Company."

Title: Unique features of ARCUS nucleases enable high efficiency, targeted gene insertion *in vivo*

Poster Number: #P641

Presenter: Cassie Gorsuch, VP of Gene Therapy, Precision Biosciences

Date and Time: Wednesday, October 25, 2023, 5:00 PM - 6:15 PM CEST and Thursday, October 26, 2023, 8:30 PM - 9:30 PM CEST

Location: Gare Maritime

In preclinical work presented today, ARCUS showed high efficiency gene insertion *in vitro* (primary human T cells and hepatocytes) and *in vivo* (infant and adult NHPs). ARCUS' 3' overhangs were shown to drive high efficiency gene insertion compared to blunt cuts, and *in vivo* ARCUS demonstrated high efficiency and durable insertion in newborn and infant NHPs when used with OTC and F9 transgene insertion templates after adeno-associated virus (AAV) delivery. ARCUS showed up to ~45% insertion when administered by LNP along with an AAV carrying a gene insertion template in adult NHPs, and demonstrated high efficiency gene insertion via HDR in nondividing, primary human hepatocytes.

Title: ARCUS-mediated excision of the "hot spot" region of the human dystrophin gene results in functional improvement in a mouse model of Duchenne muscular dystrophy (DMD)

Poster Number: Poster #P653

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In the data on display today using early generation ARCUS nucleases, Precision scientists observed the edited dystrophin protein variant in multiple tissue types frequently involved in progression of DMD, including heart, diaphragm, and skeletal muscle. Furthermore, the maximum force output of the gastrocnemius muscle in ARCUS-treated animals was significantly improved compared to untreated mice, reaching 86% of the maximum force output levels observed in non-diseased, control animals.

About Duchenne muscular dystrophy

DMD is a genetic disorder associated with mutations in the dystrophin gene that prevent production of the dystrophin protein. Dystrophin stabilizes the cell membrane during muscle contraction to prevent damage, and the absence of intact dystrophin protein leads to inflammation, fibrosis, and progressive loss of muscle function and mass. Over time, children with DMD will develop problems walking and breathing, eventually leading to death in the second or third decade of life due to progressive cardiomyopathy and respiratory insufficiency. DMD occurs in 1 in 3,500 to 5,000 male births, and currently there are limited approved therapies available for patients.

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), excise (knock-out), eliminate, 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 and stimulate gene insertion at the cut site by homologous recombination. Precision's platform and products are protected by a comprehensive portfolio including nearly 100 patents to date.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is an advanced gene editing company dedicated to improving life (DTIL) with its novel and proprietary ARCUS® genome editing platform that differs from other technologies in the way it cuts, its smaller size, and its simpler structure. Key capabilities and differentiating characteristics may enable ARCUS nucleases to drive more intended, defined therapeutic outcomes. Using ARCUS, the Company's pipeline is comprised of in vivo gene editing candidates designed to deliver lasting cures for the broadest range of genetic and infectious diseases where no adequate treatments exist. For more information about Precision BioSciences, please visit 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 expected conference participation and disclosure of preclinical data, the ability of ARCUS to make predictable, highly consistent, therapeutic edits as well as durable, high-efficiency gene insertion capabilities through HDR, including NHPs, the potential to redefine gene editing by enabling sophisticated gene edits such as gene insertion, excision, and elimination, the clinical development, nomination, and goals of our PBGENE-DMD program, therapeutic potential of an ARCUS gene editing approach for the treatment of DMD, including ability of ARCUS to achieve large gene excisions, enabling significant functional muscle improvement in a DMD mouse model, and expected safety, efficacy, and benefit of our gene editing approaches. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should," "target," "will," "would," 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, the important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, 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 page of our website under SEC Filings 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.

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