Precision BioSciences Presents Preclinical Data Demonstrating Potential of ARCUS for Treatment of Duchenne Muscular Dystrophy at the American Society of Gene & Cell Therapy 26th Annual Meeting

May 19, 2023

Late-breaking, oral presentation highlights ability of ARCUS gene editing approach to achieve large gene excisions enabling significant functional muscle improvement in preclinical study

DURHAM, N.C.--(BUSINESS WIRE)--May 19, 2023-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company developing ARCUS®-based *in vivo* gene editing and *ex vivo* allogeneic CAR T therapies, today announced that the company will present preclinical data demonstrating the potential of ARCUS *in vivo* gene editing for large gene excisions toward the goal of treating Duchenne muscular dystrophy (DMD) at the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting. The oral presentation, titled *"ARCUS-Mediated Excision of the "Hot Spot" Region of the Human Dystrophin Gene for the Treatment of Duchenne Muscular Dystrophy (DMD),"* will be delivered during the Late-breaking Abstracts 2 Session held today from 10:15 AM to 12:00 PM PT.

"While traditional gene therapies have shown promise in potentially slowing or stabilizing the progression of DMD, there remains no curative therapy for this disorder," said Jeff Smith, Chief Research Officer of Precision BioSciences. "During today's presentation, we are excited to share the first *in vivo* data demonstrating the therapeutic potential of an ARCUS gene editing approach for DMD, which may one day enable the single administration of a drug with life-long benefits of muscle retention and function to a broad patient population."

Precision's PBGENE-DMD program strategy is to restore expression of a functional form of dystrophin by utilizing a pair of ARCUS nucleases that are delivered by a single adeno-associated virus (AAV) to excise an approximately 500,000 base pair mutation "hot spot" region of the dystrophin gene, resulting in a variant of the dystrophin protein that is functionally competent. Up to 50% of DMD patients have pathogenic mutations in this region, suggesting this editing strategy could have broad applicability compared to mutation-specific approaches.

In the data reported today using early generation ARCUS nucleases, scientists observed the edited dystrophin variant in multiple tissue types frequently involved in progression of DMD, including skeletal muscle, heart, and diaphragm. 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.

"We believe that Precision's approach to DMD is differentiated by the potential of ARCUS nucleases to precisely excise large genomic regions and repair the gene with high efficiency," said Cassie Gorsuch, VP of Gene Therapy at Precision. "ARCUS nucleases have the unique capability to generate 4 base pair 3' overhangs, or 'sticky ends,' following DNA cleavage. In our PBGENE-DMD program, we've engineered a pair of ARCUS nucleases that generate complementary overhangs at their target sites in the dystrophin gene to promote perfect re-ligation after excision of the 'hot spot' region. Due to the small size of ARCUS nucleases, we are able to deliver both using a single AAV." The *in vivo* proof-of-concept study presented today demonstrates the therapeutic potential of an ARCUS gene editing approach for the treatment of DMD and highlights the unique advantages of the ARCUS gene editing platform.

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), 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 a clinical stage biotechnology company dedicated to improving life (DTIL) with its novel and proprietary ARCUS® genome editing platform. ARCUS is a highly precise and versatile genome editing platform that was designed with therapeutic safety, delivery, and control in mind. Using ARCUS, the Company's pipeline consists of several *in vivo* gene editing candidates designed to cure genetic and infectious diseases where no adequate treatments exist and multiple *ex vivo* clinical candidates. 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 clinical development, nomination, and goals of our PBGENE-DMD program, therapeutic potential of an ARCUS gene editing approach for the treatment of DMD, and expected safety, efficacy, and benefit of our gene editing approaches including re-ligation editing efficiency. 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 neither promises nor guarantees, but involve 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 quarterly period ended March 31, 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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Source: Precision BioSciences, Inc.