Precision BioSciences Announces In Vivo Gene Editing Collaboration with Novartis to Develop Potentially Curative Treatment for Disorders Including Sickle Cell Disease

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- Precision to Receive $75 Million Upfront for a Single Target; Precision Eligible to Receive up to an Additional $1.4 Billion in Milestones and Tiered Royalties on Sales of Licensed Products

- Precision to Develop a Single ARCUS® Nuclease Designed for Safe and Efficient In Vivo Gene Insertion

- Collaboration Combines Precision's Proprietary ARCUS Genome Editing Platform and Gene Insertion Capabilities with Novartis' Drug Discovery and Gene Therapy Expertise

- Extends Precision's Cash Runway into Q2 2024

- Precision to Host Conference Call Tomorrow, June 22, 2022, at 8:00 AM ET

DURHAM, N.C.--(BUSINESS WIRE)--Jun. 21, 2022-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company developing ARCUS-based *ex vivo* allogeneic CAR T and *in vivo* gene editing therapies, today announced it has entered into an exclusive worldwide *in vivo* gene editing research and development collaboration and license agreement with Novartis Pharma AG (the "Agreement"). As part of the Agreement, Precision will develop a custom ARCUS nuclease that will be designed to insert, *in vivo,* a therapeutic transgene at a "safe harbor" location in the genome as a potential one-time transformative treatment option for diseases including certain hemoglobinopathies such as sickle cell disease and beta thalassemia.

Under the terms of the Agreement, Precision will develop an ARCUS nuclease and conduct *in vitro* characterization, with Novartis then assuming responsibility for all subsequent research, development, manufacturing and commercialization activities. Novartis will receive an exclusive license to the custom ARCUS nuclease developed by Precision for Novartis to further develop as a potential *in vivo* treatment option for sickle cell disease and beta thalassemia. Precision will receive an upfront payment of $75 million and is eligible to receive up to an aggregate amount of approximately $1.4 billion in additional payments for future milestones. Precision is also eligible to receive certain research funding and, should Novartis successfully commercialize a therapy from the collaboration, tiered royalties ranging from the mid-single digits to low-double digits on product sales.

"We are excited to collaborate with Novartis to bring together the precision and versatility of ARCUS genome editing with Novartis' gene therapy expertise and commitment to developing one-time, potentially transformative treatment for hard-to-treat inherited blood disorders," said Michael Amoroso, Chief Executive Officer at Precision BioSciences. "This collaboration will build on the unique gene insertion capabilities of ARCUS and illustrates its utility as a premium genome editing platform for potential *in vivo* drug development. With this Agreement, Precision, either alone or with world-class partners, will have active *in vivo* gene editing programs for targeted gene insertion and gene deletions in hematopoietic stem cells, liver, muscle and the central nervous system showcasing the distinctive versatility of ARCUS."

"We identify here a collaborative opportunity to imagine a unique therapeutic option for patients with hemoglobinopathies, such as sickle cell disease and beta thalassemia – a potential one-time treatment administered directly to the patient that would overcome many of the hurdles present today with other therapeutic technologies," said Jay Bradner, President of the Novartis Institutes for Biomedical Research (NIBR), the Novartis innovation engine. "We look forward to working with Precision and leveraging the ARCUS technology platform, which could bring a differentiated approach to the treatment of patients with hemoglobinopathies."

"The *in vivo* gene editing approach that we are pursuing for sickle cell disease could have a number of significant advantages over other *ex vivo* gene therapies currently in development," said Derek Jantz, Ph.D., Chief Scientific Officer and Co-Founder of Precision BioSciences. "Perhaps most importantly, it could open the door to treating patients in geographies where stem cell transplant is not a realistic option. We believe that the unique characteristics of the ARCUS platform, particularly its ability to target gene insertion with high efficiency, make it the ideal choice for this project, and we look forward to working with our partners at Novartis to bring this novel therapy to patients."

Upon completion of the transaction, Precision expects that existing cash and cash equivalents, expected operational receipts, and available credit will be sufficient to fund its operating expenses and capital expenditure requirements into Q2 2024.

Precision BioSciences Conference Call and Webcast Information

Precision's management team will host a conference call and webcast tomorrow, June 22, 2022, at 8:00 AM ET to discuss the collaboration. The dial-in conference call numbers for domestic and international callers are (866)-996-7202 and (270)-215-9609, respectively. The conference ID number for the call is 6252688. Participants may access the live webcast on Precision's website https://investor.precisionbiosciences.com/events-and-presentations in the Investors page under Events and Presentations. An archived replay of the webcast will be available on Precision's website.

About ARCUS and "Safe harbor" ARCUS Nucleases

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 nearly 100 patents to date.

Precision can use an ARCUS nuclease to add a healthy copy of a gene (or "payload") to a person's genome. The healthy copy of the gene can be inserted at its usual site within the genome, replacing the mutated, disease-causing copy. Alternatively, an ARCUS nuclease can be used to insert a healthy copy of the gene at another site within the genome called a "safe harbor" that enables production of the healthy gene product without otherwise affecting the patient's DNA of gene expression patterns.

About Sickle Cell Disease and Beta Thalassemia

Sickle cell disease (SCD) is a complex genetic disorder that affects the structure and function of hemoglobin, reduces the ability of red blood cells to transport oxygen efficiently and, early on, progresses to a chronic vascular disease.[1-4] The disease can lead to acute episodes of pain known as sickle cell pain crises, or vaso-occlusive crises, as well as life-threatening complications.[5-7] The condition affects 20 million people worldwide.[8] Approximately 80% of individuals with SCD globally live in sub-Saharan Africa and it is estimated that approximately 1,000 children in Africa are born with SCD every day and more than half will die before they reach five.[9,10] SCD is also a multisystem disorder and the most common genetic disease in the United States, affecting 1 in 500 African Americans. About 1 in 12 African Americans carry the autosomal recessive mutation, and approximately 300,000 infants are born with sickle cell anemia annually.[11] Even with today's best available care, SCD continues to drive premature deaths and disability as this lifelong illness often takes an extreme emotional, physical, and financial toll on patients and their families.[12,13]

Beta thalassemia is also an inherited blood disorder characterized by reduced levels of functional hemoglobin.[14] The condition has three main forms – minor, intermedia and major, which indicate the severity of the disease.[14] While the symptoms and severity of beta thalassemia varies greatly from one person to another, a beta thalassemia major diagnosis is usually made during the first two years of life and individuals require regular blood transfusions and lifelong medical care to survive.[14] Though the disorder is relatively rare in the United States, it is one of the most common autosomal recessive disorders in the world.[14] The incidence of symptomatic cases is estimated to be approximately 1 in 100,000 individuals in the general population.[14, 15] The frequency of beta-thalassemia mutations varies by regions of the world with the highest prevalence in the Mediterranean, the Middle-East, and Southeast and Central Asia. Approximately 68,000 children are born with beta-thalassemia.[16]

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 multiple *ex vivo* "off-the-shelf" CAR T immunotherapy clinical candidates and several *in vivo* gene editing candidates designed to cure 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, as may any related presentations, within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this herein and in any related presentation that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the goal of providing a one time, potentially curative treatment for certain hemoglobinopathies, the success of the collaboration with Novartis, including the receipt of any milestone, royalty, or other payments pursuant to and the satisfaction of obligations under the Agreement, clinical and regulatory development and expected efficacy and benefit of our platform and product candidates, expectations about our operational initiatives and business strategy, expectations about achievement of key milestones, and expected cash runway. 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," "potential," "predict," "project," "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: 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 studies and clinical trials; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, and 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 COVID-19 pandemic and variants thereof, 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 March 31, 2022, 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.

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