BridgeBio Pharma Announces Dosing of First Patient in Phase 1/2 Trial of Investigational Gene Therapy for Congenital Adrenal Hyperplasia (CAH)

- If successful, BridgeBio's investigational gene therapy BBP-631 would be the first therapy for CAH to restore the body's hormone and steroid balance by enabling people with CAH to make their own cortisol and aldosterone

-Initial Phase 1/2 data readout anticipated in the second half of 2022

-BridgeBio's gene therapy portfolio also includes a clinical stage program for Canavan disease and preclinical programs for classic galactosemia, TMC1 hearing loss, tuberous sclerosis types 1 and 2, cystinuria and a genetic dilated cardiomyopathy

PALO ALTO, CA – January 27, 2022 — BridgeBio Pharma, Inc. (Nasdaq: BBIO) ("BridgeBio" or the "Company"), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today announced that the first patient has been dosed in ADventure, its Phase 1/2 clinical trial of BBP-631, an investigational adeno-associated virus (AAV) 5 gene therapy for the treatment of classic congenital adrenal hyperplasia (CAH). CAH is one of the most prevalent genetic diseases, with more than 75,000 cases estimated in the United States and European Union.

"Dosing the first patient in our CAH trial is a landmark milestone and we are grateful for the support from the medical and patient communities who helped us reach this moment. For more than 50 years people living with CAH have had the same limited standard of care – lifelong daily steroid replacement treatment. Our investigational gene therapy offers patients a potential single-dose intervention designed to restore their body's hormone and steroid balance by making their own cortisol and aldosterone," said Eric David, M.D., J.D., CEO at BridgeBio Gene Therapy. "This is the second gene therapy trial we have initiated in less than four months, and we are excited to advance this trial and our other gene therapy programs in the hope of improving patients' lives."

"Adults, children and families affected by CAH experience the daily burden of the disease and often, unfortunately, the side effects and morbidities associated with the current treatment regimens. As an endocrinologist, it's incredibly exciting to reimagine a new approach to treating this disease," added Adam Shaywitz, M.D., Ph.D., chief medical officer at BridgeBio Gene Therapy. Adrenas Therapeutics, the affiliate company of BridgeBio focused on developing BBP-631 for CAH, is part of BridgeBio Gene Therapy's portfolio.

The Phase 1/2 open-label study is designed to evaluate the safety, tolerability and pharmacodynamic activity of the company's AAV5 gene therapy, BBP-631, in adults with classic CAH. In the initial dose-finding phase of the study, each subject will receive a single intravenous (IV) infusion. The primary outcomes of the study are safety, as well as change from baseline in endogenous cortisol levels, which BBP-631 has the unique potential to restore. Change from

baseline in steroid biomarkers for hydroxyprogesterone (17-OHP) levels and androstenedione (A4) levels will also be measured. Preclinical proof-of-concept data have shown the approach provides efficient and persistent delivery of functional 21-hydroxylase (21-OH) enzyme to the adrenal gland.

“We are honored to be the first site to administer gene therapy in a patient with CAH as it is a potential game-changing treatment option that targets the disease at its source,” said Kyriakie Sarafoglou, M.D., associate professor and director of the Center for Congenital Adrenal Hyperplasia at the University of Minnesota. “We are eager to see whether gene therapy can restore endogenous cortisol production, and also look forward to exploring its effect on the physiological mechanisms that regulate the hypothalamic-pituitary-adrenal axis including circadian and ultradian hormonal profiles.”

BridgeBio’s investigational AAV5 gene therapy for CAH is one of the Company’s 14 programs that are in the clinic or commercial setting for patients living with genetic diseases and cancers. Initial Phase 1/2 data readouts of the Company’s AAV5 gene therapy for CAH and the Company’s AAV9 gene therapy for Canavan disease are expected in the second half of 2022.

For more information about the ADventure trial, visit ClinicalTrials.gov (NCT04783181).

About BBP-631
BBP-631 is an AAV5 gene therapy developed to treat CAH due to 21-hydroxylase deficiency at its source. BBP-631 is designed to deliver a functional copy of the 21-hydroxylase gene and has been shown through multiple preclinical studies to result in efficient and persistent delivery to the adrenal gland, where hormones are naturally made. If successful**,** BBP-631 may restore the body’s hormone and steroid balance by enabling people with CAH to naturally make their own cortisol and aldosterone. It could also allow for people with CAH to eliminate or significantly reduce their daily glucocorticoid or mineralocorticoid doses, which is the current standard of care for patients.

About Congenital Adrenal Hyperplasia (CAH)
Affecting approximately 75,000 people in the United States and European Union, CAH is a group of genetic disorders that affect the adrenal glands, which is caused by a mutation in the gene encoding for 21-hydroxylase, an enzyme essential for making the hormones cortisol and aldosterone which are critical for various physiologic functions. Cortisol is necessary for the body to respond to injury, stress or illness, and aldosterone is required to maintain proper blood pressure and sodium levels. Unable to produce cortisol and aldosterone, people with classic CAH cannot mount the healthy physiological response to stressors, such as illnesses, that allows their heart, lungs, kidneys and other organs to compensate for the stress, which can be life-threatening. These adrenal crises can be particularly dangerous for young children.

About BridgeBio Pharma, Inc.
BridgeBio Pharma, Inc. (BridgeBio) is a commercial-stage biopharmaceutical company founded to discover, create, test and deliver transformative medicines to treat patients who suffer from

genetic diseases and cancers with clear genetic drivers. BridgeBio's pipeline of over 30 development programs ranges from early science to advanced clinical trials and its commercial organization is focused on delivering the company's first two approved therapies. BridgeBio was founded in 2015 and its team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit bridgebio.com and follow us on LinkedIn and Twitter.

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), which are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements relating to the timing and success of BridgeBio's Phase 1/2 clinical trial of BBP-631 for the treatment of CAH, expectations, plans and prospects regarding BridgeBio's regulatory approval process for BBP-631, the ability of BBP-631 to treat CAH in humans, and the timing and success of initial top-line Phase 1/2 date of BBP-631, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a number of risks, uncertainties and assumptions, including, but not limited to, BridgeBio's ability to continue and complete its Phase 1/2 clinical trial of BBP-631 for the treatment of CAH, past data from preclinical studies not being indicative of future data from clinical trials, BridgeBio's ability to advance BBP-631 in clinical development according to its plans, the ability of BBP-631 to treat CAH, the ability of BBP-631 to retain Fast Track Designation, Rare Pediatric Drug Designation, and Orphan Drug Designation from the U.S. Food and Drug Administration and Orphan Drug Designation from the European Medicines Agency, risks inherent in developing therapeutic products, the success, cost, and timing of the Company's product candidate research and development activities and ongoing and planned preclinical studies and clinical trials, the success and timing of preclinical study and clinical trial results, the success of its clinical trial designs, the fact that successful preliminary preclinical study or clinical trial results may not result in future clinical trial successes and/or product approvals, trends in the industry, the legal and regulatory framework for the industry, the success of current and future agreements with third parties in connection with the development or commercialization of the Company's product candidates and FDA-approved products, the size and growth potential of the market for the Company's product candidates

and FDA-approved products, the Company's ability to access additional funding upon achievement of portfolio milestones, the accuracy of the Company's estimates regarding expenses, future revenue, future expenditures and needs for and ability to obtain additional financing, the Company's ability to be a sustainable genetic medicine innovation engine and to build the next great genetic medicine company, the Company's ability to obtain and maintain intellectual property protection for its product candidates and approved products, the competitive environment and clinical and therapeutic potential of the Company's product candidates and FDA-approved products, the Company's international expansion plans, and potential adverse impacts due to the global COVID-19 pandemic such as delays in clinical trials, preclinical work, overall operations, regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy; as well as those set forth in the Risk Factors section of BridgeBio's most recent Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission (SEC) and in subsequent SEC filings, which are available on the SEC's website at **www.sec.gov**. Moreover, BridgeBio operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. Except as required by applicable law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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