NEWS RELEASE

bridgebio pharma receives fda fast track designation for investigational gene therapy for congenital adrenal hyperplasia

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- Preclinical Data for Congenital Adrenal Hyperplasia and Canavan Disease Programs Shared at American Society of Gene & Cell Therapy Annual Meeting, Enabling Anticipated Clinical Trials This Year

PALO ALTO, Calif., May 14, 2021 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO), a commercial-stage biopharmaceutical company founded to discover, create, test and deliver meaningful medicines for patients with genetic diseases and cancers with clear genetic drivers, today announced that the U.S. Food and Drug Administration (FDA) granted Fast Track designation to BBP-631, an investigational adeno-associated virus 5 (AAV5) gene therapy designed for the treatment of congenital adrenal hyperplasia (CAH).

Fast Track designation, granted by the FDA, is designed to facilitate the development and to expedite the review of new therapies hoping to treat or prevent serious conditions and fill an unmet medical need. Additionally, BBP-631 was granted Rare Pediatric Disease Designation by the FDA and has received Orphan Drug Designation by the FDA and European Medicines Agency (EMA).

“The standard-of-care for CAH patients has not changed significantly over the last 50 years, and a gene therapy offers for the first time the possibility that patients may be able to make their own cortisol and aldosterone, at the right times and in the right amounts. The FDA’s Fast Track designation reinforces the urgency to address the unmet needs of patients with CAH as quickly and safely as possible. We are grateful to have received Fast Track along with other key designations granted by the FDA and the EMA,” said Eric David, M.D., J.D., CEO at BridgeBio Gene Therapy, which is focused on developing gene therapy treatment options for patients in need. “We are eager to initiate our

first-in-human Phase 1/2 study. The Investigational New Drug application has been cleared by the FDA and site activation for the study is ongoing, and is expected to begin in the coming months."

CAH is one of the most prevalent genetic diseases with more than 75,000 cases estimated in the United States and Europe. The disease is caused by deleterious mutations in the gene encoding an enzyme called 21-hydroxylase, leading to lack of endogenous cortisol and aldosterone production. This lack of production causes patients with CAH to be unable to form physiological responses to illnesses and stressors, which can be life-threatening, especially for children. BBP-631 is designed to provide a functional copy of the 21-hydroxylase-encoding gene to help patients produce their own cortisol and aldosterone.

Additionally, BridgeBio Gene Therapy presented preclinical data from its CAH program on May 13, 2021 at the American Society of Gene & Cell Therapy (ASGCT) annual meeting, being held virtually May 11-14, 2021. The presentation covered three key Investigational New Drug (IND)-enabling studies that informed the anticipated upcoming clinical trial for BBP-631. The IND application has been cleared by the FDA and site activation for initiation of a first-in-human Phase 1/2 study is ongoing, with initial data anticipated in late 2021 or early 2022. At ASGCT, BridgeBio Gene Therapy today will also be presenting preclinical data from its program designed for the treatment of Canavan disease, which is an extremely rare genetic disease starting in infancy with an incidence of approximately one in 100,000 births worldwide.

"ASGCT is an important opportunity to share the robust preclinical data package underlying our programs. We hope that by rapidly advancing our investigational therapies into the clinic, we will be one step closer to providing CAH and Canavan patients with potential therapeutic relief," said Clayton Beard, senior vice president of research and development at BridgeBio Gene Therapy.

ASGCT Oral Presentations:

Intravenous AAV5 Gene Therapy with Human CYP21A1 Corrects Phenotypic Deficiencies of the 21-Hydroxylase Knockout Mouse Model and Demonstrates Durability and Safety in Non-Human Primates and Mice
Presenter: Rachel Eclov, Ph.D., associate director of preclinical gene therapy at BridgeBio Gene Therapy
Presentation date/time: Thursday May 13, 2021 at 6:45 – 7:00 p.m. ET

- The preclinical data presented shows dose-dependent efficacy, dose-dependent biodistribution and no safety concerns in the shared studies.
- Results from all three studies shared in the presentation support the efficacy and safety for the upcoming Phase 1/2 trial in classic CAH.

Safety Evaluation of IV-Administered BBP-812, an AAV9-Based Gene Therapy for the Treatment of Canavan Disease,

in Mice and Juvenile Cynomolgus Macaques
Presenter: David Scott, director of nonclinical pharmacology and toxicology at BridgeBio Gene Therapy
Presentation date/time: Friday, May 14, 2021 at 2:00 – 2:15 p.m. ET

- The preclinical findings demonstrate safety through IV administration, which will provide superior biodistribution to deep brain regions and avoid potentially invasive brain surgery.
- The toxicology study shows safety and facilitates a transition into IND and the clinic to begin testing in patients with Canavan disease.

For more information on the upcoming clinical trial in CAH, please visit clinicaltrials.gov (Identifier: **NCT04783181**).

About BridgeBio Pharma, Inc.
BridgeBio Pharma (BridgeBio) is a 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 approved therapy. 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**.

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 expectations, plans and prospects regarding the preclinical and clinical development plans, clinical trial designs, clinical and therapeutic potential, and strategy of our product candidates, including, but not limited to: the unknown future impact of the COVID-19 pandemic delay on our preclinical and clinical development plans and/or our operations or operating expenses; the timing and success of our planned preclinical and clinical development of our development programs, including each of BBP-812 and BBP-631; the timing and success of any such continued preclinical and clinical development and planned regulatory submissions, including for each of BBP-812 and BBP-631; the potential therapeutic and clinical benefits of each of BBP-812 and BBP-631; the potential size of the target patient populations for each of BBP-812 and BBP-631; the potential for BBP-812 to be the first approved therapy for CAH and for BBP-631 to be the first approved therapy for Canavan

disease; our expected runway for cash, cash equivalents and marketable securities; and the timing of these events, 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: the success of clinical trials, regulatory filings, approvals and/or sales; despite having ongoing interactions with the FDA or other regulatory agencies, the FDA or such other regulatory agencies may not agree with our regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted; potential adverse impacts due to the global COVID-19 pandemic such as delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy; and those risks set forth in the Risk Factors section of our most recent quarterly or annual periodic report filed with the SEC and our other SEC filings. Moreover, BridgeBio operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of BridgeBio's management as of the date of this release and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. 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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