NEWS RELEASE

bridgebio pharma reports topline results from phase 1/2 trial of investigational gene therapy for congenital adrenal hyperplasia (cah)

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- Increase in endogenous cortisol production achieved in all patients in higher dose cohorts of BBP-631, a result seen for the first time ever in CAH patients

- The gene therapy was well tolerated with no treatment-related serious adverse events (SAEs) reported

- Despite novel scientific advancements achieved with this program, the data do not warrant additional capital investment at this time and the gene therapy budget is being significantly reduced

PALO ALTO, Calif., Sept. 10, 2024 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (“BridgeBio” or the “Company”), a commercial-stage biopharmaceutical company focused on genetic diseases, today announced topline results from the Phase 1/2 open-label ADventure study investigating BBP-631, the Company’s investigational adeno-associated virus (AAV) 5 gene therapy, for the treatment of congenital adrenal hyperplasia (CAH).

The Phase 1/2 open-label ADventure study was designed to evaluate the safety, tolerability and pharmacodynamic activity of BBP-631 in adults with classic CAH. To date, key results from the study include:

- Increased endogenous cortisol production was achieved in all patients at higher doses. A maximum change from baseline post-ACTH stimulation test of 4.7 µg/dL and 6.6 µg/dL was observed at the two highest dose levels, respectively, with cortisol levels as high as 11 µg/dL achieved.
- Substantial and durable increases in 11-deoxycortisol, the product of 21-hydroxylase, and reductions in 17-

hydroxyprogesterone (17-OHP), the substrate of 21-hydroxylase, provide compelling evidence of durable BBP-631 transgene activity.
    - At the highest dose levels, sustained 11-deoxycortisol averaged a 55-fold increase from baseline with a maximum of 99-fold increase from baseline. These represent an average maximum of 23-fold the upper-limit of normal.
    - Robust reduction in 17-hydroxyprogesterone, with the majority of patients reaching a reduction of ≥50%, with a max reduction of 95%.
- BBP-631 has been well tolerated with only mild to moderate treatment-emergent adverse events (TEAEs) and no treatment-related SAEs reported.

“While the data to date are not yet transformational, the study showed for the first time that people living with CAH can indeed make their own cortisol, and that gene therapy can be safely administered in this patient population. We remain committed to finding the right partner for those in the CAH community and are grateful to the participants and those who expressed interest in both the pre-screening study and the ADventure study. We also want to thank the ADventure study investigators and staff, the CAH patient advocacy organizations and the broader CAH community,” said Neil Kumar, Ph.D., CEO and Founder of BridgeBio.

“Given that the results of the trial did not meet the threshold to warrant additional capital investment at this time, BridgeBio will be reducing the gene therapy budget more than $50M, consistent with our capital allocation principles, and reserving gene therapy for priority targets that we cannot treat any other way,” said Brian Stephenson, Ph.D., CFA, Chief Financial Officer of BridgeBio. “We believe that gene therapies have the potential to fulfill a significant unmet need and are eager to work closely with the FDA and the Canavan community with the goal of bringing our therapy to families living with Canavan disease as fast as possible.”

BridgeBio will no longer be pursuing development of BBP-631 for CAH and the Company is actively seeking partnership opportunities to support future development of BBP-631 or next-generation gene therapies for the treatment of CAH, a very prevalent genetic disease that still has significant unmet need, with more than 75,000 cases estimated in the United States and European Union.

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. BridgeBio's pipeline of development programs ranges from early science to advanced clinical trials. 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**, **Twitter** and **Facebook**.

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements BridgeBio makes 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," "continues," "estimates," "expects," "hopes," "intends," "may," "plans," "projects," "remains," "seeks," "should," "will," and variations of such words or similar expressions. BridgeBio intends 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. These forward-looking statements, including statements in Dr. Kumar's quote relating to the expectations, plans and prospects regarding BBP-631, the statements in Dr. Stephenson's quote relating to the Company's financial performance, capitalization status, strategy, business plans and goals and the potential for gene therapy and future partnership opportunities for 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 BridgeBio believes that its plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, BridgeBio 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, 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 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, potential adverse impacts due to global health emergencies, including delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, the impacts of current macroeconomic and geopolitical events, including changing conditions from hostilities in Ukraine and in Israel and the Gaza Strip, increasing rates of inflation and rising interest rates, on our business operations and expectations as well as those risks set forth in the Risk Factors section of BridgeBio's most recent Annual Report on Form 10-K, and BridgeBio's other filings with the U.S. Securities and Exchange Commission. 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 press 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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