NEWS RELEASE

bridgebio pharma presents positive phase 1 data in healthy volunteers, advancing development of bbp-671 for pantothenate kinase-associated neurodegeneration (pkan) and organic acidemias

2022-05-26

- BBP-671 detected in healthy volunteer plasma and cerebrospinal fluid (CSF), suggesting that BBP-671 is entering the brain, a location critical to target neurological complications of PKAN and organic acidemias at their source

- Results showed BBP-671 increased whole blood acetyl-coenzyme-A (CoA) levels, a signal supporting proof of mechanism of the therapy

- Based on these data, BridgeBio intends to move forward with the second part of the Phase 1 clinical study in patients with propionic acidemia and methylmalonic acidemia in the second half of 2022, and plans to initiate a pivotal Phase 2/3 study in PKAN in 2023

- Data presented in a scientific poster session at the Pan American Parkinson and Movement Disorders (PAS) Congress

PALO ALTO, Calif., May 26, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (“BridgeBio” or the “Company”), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today announced interim Phase 1 data from healthy volunteers, supporting the development of BBP-671 for potential treatment of pantothenate kinase-associated neurodegeneration (PKAN) and organic acidemias. The data are being shared in a scientific poster session at the Pan American Parkinson and Movement Disorders (PAS) Congress on

May 26, 2022, in Miami, FL.

BBP-671 is an investigational oral therapy designed to increase coenzyme-A (CoA) levels by allosterically modulating pantothenate kinases, key enzymes in the CoA biosynthesis pathway. BBP-671 is being developed as a potential therapy for diseases in which CoA metabolism is deficient, including PKAN, propionic acidemia (PA), and methylmalonic acidemia (MMA).

PKAN is a rare neurological disorder characterized by progressively debilitating symptoms that begin in early childhood, including dystonia, rigidity, bradykinesia, dysphagia, visual impairment and dementia. PA and MMA are rare metabolic disorders caused by mutations in enzymes that participate in amino acid metabolism leading to life-threatening metabolic decompensations, as well as long-term complications involving multiple organ systems, including the heart, pancreas, kidney, liver and brain. PKAN, PA and MMA are rare diseases affecting an estimated 7,000 patients in the United States and European Union collectively. BridgeBio believes these initial findings from the Phase 1 study demonstrate target engagement and proof of mechanism of BBP-671 provided by evidence that BBP-671 can cross the blood brain barrier.

“Given the severe unmet need for these patients, we were very encouraged by the elevation in whole blood acetyl-CoA in response to BBP-671 administration, which we believe is the first time this has been demonstrated in humans with a therapeutic intervention,” said Agnieszka Jurecka, M.D., Ph.D., vice president of clinical development at BridgeBio. “It was also critical to establish that BBP-671 crosses the blood brain barrier since PKAN is a neurodegenerative disease and PA and MMA patients suffer frequent neurological complications. We are pleased to achieve this important milestone and bring BBP-671 one step closer to patients.”

The first-in-human Phase 1 study of BBP-671 in a total of 77 healthy volunteers was designed to provide single- and multiple dose safety, tolerability, pharmacokinetic (PK), and pharmacodynamic (PD) data to support future development of BBP-671. Single and multiple ascending oral doses of BBP-671 were administered in a double-blind manner to six single and four multiple dose cohorts consisting of up to eight healthy volunteers per cohort. The data from the first part of the Phase 1 study included:

- BBP-671 was detected in healthy volunteer plasma and cerebrospinal fluid (CSF), which suggests BBP-671 is entering into the brain
- BBP-671 increased whole blood acetyl-CoA levels, which BridgeBio believes is the first time a therapeutic intervention has demonstrated this and demonstrates target engagement and proof of mechanism of the drug
- BBP 671 was generally well-tolerated following oral dosing in healthy volunteers with no serious adverse events observed
- Mild treatment-related emergent adverse events included headache, abdominal pain and nausea (5%, 1.7%

and 1.7%, respectively, of all individuals treated in single ascending dose and multiple ascending dose with BBP-671)

- Asymptomatic neutropenia was observed in three individuals with repeat dosing of BBP-671; all returned to within normal limits within a few days without any sequelae upon cessation of therapy

Based on these positive data, BridgeBio intends to move forward with the second part of its Phase 1 clinical study in patients with PA and MMA in the second half of 2022, as well as initiate a pivotal Phase 2/3 clinical study in PKAN in 2023.

More information about the ongoing Phase 1 clinical trial of BBP-671 (study number NCT04836494) can be found **here** on the ClinicalTrials.gov website. To review the scientific poster presented at the PAS Congress, please visit "Presentations & Publications" within the "Science & Pipeline" section of the BridgeBio website at **https://bridgebio.com/publications/**.

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 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** 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 prospects of success for the development of BBP-671, including plans to move forward with the second part the Phase 1 clinical study in patients with propionic acidemia and methylmalonic acidemia in the second half of 2022, as well as initiate a pivotal Phase 2/3 clinical study in PKAN in 2023, reflect our current views about our plans, intentions, expectations, strategies and prospects, and are based on the information currently available to us and on assumptions we have made and are not forecasts, promises nor guarantees. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or

suggested by these 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 our product candidates to treat genetically driven diseases and cancers with clear genetic drivers, our anticipated cash runway and our being fully funded through the completion of the BBP-671 study and our ability to access additional funding upon achievement of portfolio milestones, as well as those risks set forth in the Risk Factors section of our most recent Annual Report on Form 10-K and BridgeBio Pharma's other SEC filings. Moreover, we operate 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.

BridgeBio Contact:
Grace Rauh
Grace.rauh@bridgebio.com
(917) 232-5478