NEWS RELEASE

bridgebio pharma announces dosing of first patient in phase 1 trial of bbp-671, a potential best-in-class treatment for propionic acidemia (pa) and methylmalonic acidemia (mma)

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- Initial data readout for patients with PA and MMA expected in the first half of 2023

- Interim data from healthy individuals, reported earlier this year, showed that BBP-671 was detected in plasma and cerebrospinal fluid (CSF) at concentrations above predicted therapeutic thresholds, suggesting the compound has the potential to impact key systemic and neurological complications of PA and MMA

- BridgeBio is in active discussions with regulators and expects to launch a pivotal Phase 2/3 clinical study of BBP-671 in pantothenate kinase-associated neurodegeneration (PKAN) in 2023

- If successful, BBP-671 has potential to be a best-in-class therapy for PA, MMA, and PKAN patients, as well as the first approved oral therapy for the treatment of systemic complications caused by CoA deficiencies

PALO ALTO, Calif., Aug. 18, 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 that the first patient has been dosed in its Phase 1 clinical trial of BBP-671, an investigational oral therapy being developed for the potential treatment of conditions caused by coenzyme A (CoA) deficiencies.

BBP-671 is an investigational oral therapy intended to increase CoA levels by allosterically modulating pantothenate

kinases, key enzymes in the CoA biosynthesis pathway. It is being developed as a potential therapy for diseases in which CoA metabolism is deficient, including propionic acidemia (PA), methylmalonic acidemia (MMA), and pantothenate kinase-associated neurodegeneration (PKAN). PA, MMA, and PKAN affect an estimated 7,000 patients in the United States and European Union collectively, with PA and MMA typically diagnosed in early infancy. BBP-671 is based on breakthrough scientific developments from St. Jude Children's Research Hospital in Memphis, Tennessee.

The first-in-human Phase 1 study of BBP-671 is a single- and multiple-ascending dose study designed to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of BBP-671. The first part of the study evaluated BBP-671 in healthy individuals and the second part of the study is evaluating BBP-671 in PA and MMA patients. Positive interim data from healthy individuals were reported earlier this year.

The first patient dosed in the second part of the trial has PA. In this part of the study, up to eight PA patients and eight MMA patients will receive BBP-671. Safety and tolerability will be assessed, as well as the PK and PD of BBP-671 using validated bioanalytical assays. A wide range of disease-related biomarkers with potential clinical relevance will be monitored during the study and compared to baseline values. The potential use of biomarkers for PA and MMA as surrogate endpoints in clinical trials for metabolic diseases is a subject of active discussion among key opinion leaders in the field.[1]

"We are eager to advance the trial of BBP-671 in the hope that it will provide a positive improvement for patients, valuable data, and ultimately lead to a meaningful therapy for patients who currently have no approved treatment options," said Zineb Ammous, M.D., clinical geneticist and Medical Director of The Community Health Clinic in Topeka, Indiana, which specializes in rare genetic conditions.

PA and MMA are rare metabolic disorders caused by mutations in genes that impact the development of 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. The current standard of care includes dietary restrictions, supplementation, and sometimes liver and/or kidney transplantation but unmet need remains high due to the long-term and life-threatening impact of these diseases.

"Currently, the majority of individuals with PA and MMA are being diagnosed through newborn screening and are on a strict diet and regimen of supplements and medications. PA and MMA patients experience life-threatening metabolic decompensations, as well as many other serious complications as a result of their disease. We are hopeful that by creating a therapy designed to target the disease by modulating fundamental metabolic pathways, we may be able to treat a condition that impacts young people so early in their lives.," said Jerry Vockley, M.D., Ph.D., Chief of Genetic and Genomic Medicine and Director of the Center for Rare Disease Therapy at the University of Pittsburg Medical Center (UPMC) Children's Hospital of Pittsburgh, Pennsylvania.

Initial data from the second part of this clinical trial are expected in the first half of 2023. The company also plans to initiate a pivotal Phase 2/3 clinical study in PKAN in 2023.

“We are excited by biomarker changes observed with BBP-671 in a preclinical study using a mouse model for PA, which were associated with improved survival. Individuals diagnosed with PA and MMA and their families currently have no treatment options beyond dietary management and liver and/or kidney transplant, and we hope that BBP-671 may represent a novel therapeutic strategy for these diseases.” said Uma Sinha, Ph.D., Chief Scientific Officer of BridgeBio.

More information about the ongoing Phase 1 clinical trial of BBP-671 (study number NCT04836494) can be found **here** on the ClinicalTrials.gov website.

References

[1] Longo, N. et al. Journal of Inherited Metabolic Disease. 2022;45:2.

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 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** 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 for an initial data readout from the second part of the Phase 1 clinical study for patients with PA and MA in the first half of 2023, the expected initiation of a pivotal Phase 2/3 clinical study in PKAN in 2023, and the potential for BBP-671, if successful, to be a best-in-class therapy for PA, MMA, and PKAN patients, as well as the first

approved oral therapy for the treatment of systemic complications caused by CoA deficiencies, 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 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.

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