NEWS RELEASE

bridgebio pharma announces early positive data for bbp-812, its investigational aav9 gene therapy for canavan disease

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- These results are the first reported demonstration of rapid and robust treatment changes in key disease markers associated with the severity of disease

- Initial pharmacodynamic results for two participants show unprecedented decreases in N-acetylaspartate (NAA) in the brain and urine, suggesting the therapy is producing functional ASPA enzyme

- If successful, BridgeBio's gene therapy could be the first therapeutic option for children born with Canavan disease, a devastating and fatal neurodevelopmental disorder

PALO ALTO, Calif., June 22, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today announced promising pharmacodynamic data from the first two participants dosed in CANaspire, its Phase 1/2 clinical trial of BBP-812, an investigational intravenous (IV) adeno-associated virus serotype 9 (AAV9) gene therapy for the treatment of Canavan disease. Canavan disease is an ultra-rare and fatal disease with no approved therapies.

"Taken together, these robust decreases in urine, cerebrospinal fluid (CSF) and brain N-acetylaspartate (NAA), along with MRI signs of new myelination reported by the principal investigator are exciting and suggest we are on the right track when it comes to potentially making a difference for patients with this disease, and we look forward to gathering more data as the trial progresses," said Genevieve Laforet, M.D., Ph.D., vice president of clinical development at Aspa Therapeutics, the BridgeBio Gene Therapy affiliate company developing the gene therapy for

Canavan disease. “We are continuing to recruit and dose new participants for CANaspire and we are grateful to be able to collaborate with the advocacy organizations in the Canavan community in the pursuit of potential meaningful therapeutic advances for children with this cruel and fatal disorder.”

Data from the first two CANaspire participants show rapid and robust post-treatment decreases in NAA in urine, and importantly, in CSF and brain tissue as shown by magnetic resonance spectroscopy (MRS), to a degree not seen in available natural history data. Reduction in brain NAA is an early signal suggesting that BBP-812 administered IV has reached its intended target behind the blood-brain-barrier and is expressing functional aspartoacylase (ASPA) enzyme. There is evidence in the scientific literature that lower NAA levels are associated with milder disease. More time will be needed to see how these reductions in NAA translate to clinical outcomes.

From a safety standpoint, IV infusions of BBP-812 have been well-tolerated, and to date, no participants have experienced a treatment-related serious adverse event. BridgeBio reported:

- At Month 6 post-treatment, Participant 1 showed:
    - 77% lowering of NAA in the CSF
    - 15% reduction in NAA in brain white matter by magnetic resonance spectroscopy (MRS) imaging
    - ~50% decrease in urine NAA
- At Month 3 post-treatment, Participant 2 showed:
    - 89% reduction of NAA in CSF
    - >50% decrease in NAA in brain white matter by MRS imaging
    - 81% drop in urine NAA

“To see this biochemical change suggests that we are reaching cells critical to the disease process, a milestone in this disease. The ongoing myelination seen on MRI and the new interactions witnessed between children and their parents are both encouraging," said Florian Eichler, M.D., director of the Leukodystrophy Service and principal investigator at Massachusetts General Hospital and lead investigator of the CANaspire trial.

While the data reported here are still early and the final safety and efficacy profile of the investigational gene therapy remains to be fully established, BridgeBio believes these data show the potential of BBP-812.

“BridgeBio’s early trial results are deeply encouraging for the Canavan community,” said Orren Alperstein, president of the Canavan Foundation, whose daughter Morgan died in 1997 of Canavan disease. “These preliminary but unprecedented decreases in brain and urine NAA suggest that meaningful progress is underway for patients and their families. The thoughtful and careful approach BridgeBio is taking in this trial continues to impress me.”

Data on the first two CANaspire participants will be presented on Friday, July 8, 2022, during Research Day at the

National Tay Sachs & Allied Diseases Association Annual Family Conference in Denver, Colorado. A broader Phase 1/2 data readout, including safety and efficacy data and updates on the pharmacodynamic data, for Canavan disease is expected later in 2022.

About CANaspire

CANaspire is a Phase 1/2 open-label study designed to evaluate the safety, tolerability, and pharmacodynamic activity of BridgeBio's AAV9 gene therapy candidate, BBP-812, in pediatric patients with Canavan disease. Each eligible patient will receive a single intravenous (IV) infusion of BBP-812. The primary outcomes of the study are safety, as well as change from baseline of urine and central nervous system N-acetylaspartate (NAA) levels. Motor function and development will also be assessed.

For more information about the CANaspire trial, visit **TreatCanavan.com** or ClinicalTrials.gov (**NCT04998396**).

About BBP-812

BBP-812 is an investigational AAV9 gene therapy for Canavan disease. Using AAV gene therapy, BridgeBio seeks to deliver functional copies of the *ASPA* gene throughout the body and into the brain, potentially correcting the disease at its source. Preclinical proof-of-concept results have shown the approach restores survival and normal motor function in Canavan disease models. BBP-812 was granted Fast Track Designation, Rare Pediatric Drug Designation, and Orphan Drug Designation by the U.S. Food and Drug Administration. BBP-812 was also granted Orphan Drug Designation by the European Medicines Agency.

About Canavan Disease

Affecting approximately 1,000 children in the United States and European Union, Canavan disease is an ultra-rare, disabling and fatal disease with no approved therapy. Most children are not able to meet developmental milestones, are unable to crawl, walk, sit or talk, and die at a young age. The disease is caused by an inherited mutation of the *ASPA* gene that codes for aspartoacylase, a protein that breaks down a compound called N-acetylaspartate (NAA). Deficiency of aspartoacylase activity results in accumulation of NAA, and ultimately results in toxicity to myelin in ways that are not currently well understood. Myelin insulates neuronal axons, and without it, neurons are unable to send and receive messages as they should. The current standard of care for Canavan disease is limited to supportive therapy.

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 success of BridgeBio's Phase 1/2 clinical trial of BBP-812 for the treatment of Canavan disease, expectations, plans and prospects regarding BridgeBio's regulatory approval process for BBP-812, the ability of BBP-812 to be the first therapeutic treatment option for children born with Canavan disease, data from subsequent CANaspire participants also showing rapid and robust post-treatment decreases in NAA in urine and in CSF and brain tissue as shown by MRS, reduction in brain NAA being an early signal suggesting that BBP-812 administered IV has reached its intended target behind the blood-brain-barrier and is expressing functional ASPA enzyme, the biochemical change seen to date suggesting that the trial is reaching cells critical to the disease process, and the timing and success of final top-line Phase 1/2 date of BBP-812, including the final safety and efficacy profile of the investigational gene therapy, 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-812 for the treatment of Canavan disease, past data from preclinical studies or early data from two participants in the open-label trial not being indicative of future or final data from clinical trials, BridgeBio's ability to advance BBP-812 in clinical development according to its plans, the ability of BBP-812 to treat Canavan disease, the ability of BBP-812 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, and potential adverse impacts due to the global COVID-19 pandemic such as delays in regulatory review, manufacturing and clinical trials, 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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