NEWS RELEASE

# bridgebio pharma presents updated encouraging clinical and biomarker data from its bbp-812 canavan disease gene therapy program at the 2023 american society of gene and cell therapy (asgct) annual meeting

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- Following treatment, the N-acetylaspartate (NAA) levels of CANaspire participants are consistent with levels seen in individuals with milder Canavan disease based on findings from the Company's natural history study and reports in the scientific literature

- Sustained reductions in NAA were measured in the urine, cerebrospinal fluid (CSF), and brain of all participants and have been observed for over a year in the earliest dosed participants

- After dosing, brain magnetic resonance imaging (MRI) of participants demonstrated robust improvements in the formation of myelin, or white matter, which is essential for brain development

- Positive changes in sitting ability and head control have also been observed using multiple assessments including the Gross Motor Function Measure-88 (GMFM-88), a standardized clinical outcome measure used by physical therapists to assess movement function in children

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

PALO ALTO, Calif., May 22, 2023 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (“BridgeBio” or the “Company”), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, presented promising positive data from six 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 a rapidly progressive, rare neurological disease that affects children from birth and has no treatment options beyond supportive care. The changes in key biomarkers, brain myelination and – critically – clinical function observed in children receiving BBP-812 in our study are compelling, and as we continue to advance our program we are hopeful that we will see outcomes that make a meaningful difference in the lives of children with Canavan disease and their families,” said Genevieve Laforet, M.D., Ph.D., vice president of clinical development at Aspa Therapeutics, the BridgeBio affiliate that is developing BBP-812 for Canavan disease. “We are beyond grateful to the children and their families who are participating in CANaspire as well as the study investigators, who together are making it possible to explore the potential of BBP-812 as a therapy for Canavan disease. We intend to work with the FDA to move our program forward as rapidly as possible, including exploring available expedited programs such as accelerated approval.”

As of March 23, 2023, findings from the trial include:

- All participants showed a rapid and lasting decrease in levels of NAA, a key chemical marker elevated in children with Canavan disease, after dosing with BBP-812
    - The duration of individual participant follow-up ranged from 1 to 15 months post-treatment
    - The most recent data continue to show reductions in NAA in all participants and all compartments tested. Across individual participants, percent decreases in NAA from baseline ranged between 70% and 95% in cerebrospinal fluid (CSF), 29% and 88% in urine, and 8% and 75% in brain (by magnetic resonance spectroscopy)
- All participants in the CANaspire trial had urine NAA levels consistent with typical Canavan disease prior to receiving BBP-812. After receiving BBP-812, urine NAA levels in all participants fell to what can be considered less severe Canavan disease as reported in the scientific literature and observed in the Company’s natural history study
- MRI scans indicated the presence of improved myelination, which is essential for brain development, in the brainstem and cerebellum of all participants
    - These findings have been noted as early as 3 months after BBP-812 administration and were present at post-dosing Month 12 in the participant who has been followed the longest in the trial
- In contrast to the natural history of typical Canavan patients in which already-limited functional abilities are

lost over time, no trial participants have lost function and there have been incremental improvements in achievement of developmental milestones
    - Improvement in the GMFM-88 sitting dimension (measuring upright head control and sitting ability) has been shown to varying degrees in all participants who have been evaluated post-treatment, including one participant who has developed the ability to walk with a mobility aid and has a score on the GMFM-88 sitting dimension consistent with that of unaffected children
- BBP-812 has been shown to be generally well-tolerated to date, with a safety profile consistent with other systemically administered AAV9 gene therapies
    - The Data and Safety Monitoring Committee (DSMC), an independent panel of expert physicians and clinical trial experts, has conducted a thorough evaluation of the CANaspire trial safety data and have endorsed the Company's plan for advancement to the next dosing level

Florian Eichler, M.D., director of the leukodystrophy service at Massachusetts General Hospital, Center for Rare Neurological Disease and principal investigator of the CANaspire gene therapy clinical trial for Canavan disease, reflects on the progress of the trial: "The reduction in NAA to levels consistent with less severe Canavan disease, MRIs indicating potential improvement of myelin in the brainstem, and the measurable functional gains observed among participants, together are unlike any findings I have seen in children with Canavan disease."

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 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," "estimates," "expects," "intends," "may," "plans," "projects," "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 relating to the expectations, plans, prospects and potential benefits of BBP-812 for the treatment of Canavan disease gene therapy program, the ability of BBP-812 to be the first therapeutic treatment option for children born with Canavan disease, the safety and efficacy profile of the investigational gene therapy, the intention of BridgeBio to work with the FDA to move its program forward as rapidly as possible, including exploring accelerated approval and other expedited review pathways, and the statement in Dr. Florian Eichler's quote regarding the potential and benefits of the gene therapy program, 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 and strategies 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 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, initial and ongoing data from our preclinical studies and clinical trials not being indicative of final data, the potential size of the target patient populations our product candidates are designed to treat not being as large as anticipated, the design and success of ongoing and planned clinical trials, future regulatory filings, approvals and/or sales, despite having ongoing and future interactions with the FDA or other regulatory agencies to discuss potential paths to registration for our product candidates, the FDA or such other regulatory agencies not agreeing with our regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted, uncertainty regarding any impacts due to COVID-19, such as 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, increasing rates of inflation and rising interest rates, on business operations and expectations, as well as those risks set forth in the Risk Factors section of our Annual Report on Form 10-K for the year ended December 31, 2022 and our other filings with the U.S. Securities and Exchange Commission. Moreover, we operate 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 our 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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