NEWS RELEASE

bridgebio pharma gene therapy subsidiaries present data demonstrating potential in two rare disease indications at the european society of gene and cell therapy conference

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- Preclinical data shows promise for gene therapy candidates for congenital adrenal hyperplasia due to 21-hydroxylase deficiency (BBP-631) and Canavan disease (BBP-812)
- Natural history clinical study for Canavan disease is currently enrolling
- IND submissions for both gene therapy product candidates anticipated in 2020

SAN FRANCISCO, Oct. 22, 2019 /PRNewswire/ -- BridgeBio Pharma, Inc. (NASDAQ: BBIO) today announced that promising preclinical data from two of its gene therapy-focused subsidiaries – Adrenas Therapeutics and Aspa Therapeutics – will be presented at the European Society of Gene and Cell Therapy (ESGCT) Conference, occurring in Barcelona from October 22 to 25. In addition, the company announced that Aspa Therapeutics is currently conducting a natural history study in children with Canavan disease, CANinform.

Experimental Gene Therapy for Congenital Adrenal Hyperplasia Shows Durable Transgene Expression

Researchers from BridgeBio subsidiary Adrenas Therapeutics presented new preclinical results from gene therapy candidate BBP-631 in a poster entitled "Durable CYP21A2 Gene Therapy in Non-Human Primates for Treatment of Congenital Adrenal Hyperplasia." Throughout the study, a total of 20 non-human primates (NHPs) were treated with BBP-631 at one of three intravenous (IV) doses. Vector copy number and transgene mRNA expression in the adrenal glands were analyzed at 4 and 12 weeks post-dosing in the low- and medium-dose arms and at 12 and 24 weeks post-dosing in the high-dose arm. No dose-related adverse events were observed at any of the doses tested

at any time point.

Overall, treatment with BBP-631 resulted in high vector copy number (VCN) and mRNA expression in the adrenal gland, suggesting strong tropism and uptake of BBP-631 for the adrenal gland. In the high-dose arm, VCNs were maintained between 12 and 24 weeks. Furthermore, mRNA levels increased between 4 and 12 weeks for the medium dose arm and were consistent between 12 and 24 weeks for the high dose arm. Researchers also saw dose-dependent increases in both VCNs and mRNA levels across the three doses tested.

"Durable expression of a vector in adrenal tissue has presented challenges for the development of gene therapies for adrenal indications," said Clayton Beard, Ph.D., Senior Vice President, Research and Development at BridgeBio Gene Therapy. "The observed maintenance of the BBP-631 AAV5 vector for as long as 24 weeks in NHPs represents an important step in validating a gene therapy approach to treat 21-hydroxylase deficiency, and we anticipate filing our IND for this indication in 2020."

Canavan Disease Experimental Therapy Amenable to IV Delivery

In a poster entitled "A Route of Administration Study of BBP-812, an AAV9-based Gene Therapy for the Treatment of Canavan Disease, in Juvenile Cynomolgus Macaques," scientists from Aspa Therapeutics examined the uptake of and resulting DNA and mRNA expression for BBP-812, an experimental AAV9 vector, in three different administration routes: intravenous (IV), intrathecal (IT) or intracerebroventricular (ICV). Biodistribution of vector genomes and of transgene mRNA was evaluated throughout the central nervous system (CNS) at both 3 and 8 weeks post-administration. No dose-related adverse events were observed at any tested doses at either time period.

Using an IV route of administration, vector copy number and mRNA expression were not only detected broadly throughout the brain and spinal cord, but were generally detected at levels higher than when administered ICV or IT.

"Developing therapies for diseases like Canavan presents particular delivery challenges – namely getting the therapy into the central nervous system," said Adam Shaywitz, M.D., Ph.D., Chief Medical Officer at BridgeBio Gene Therapy. "In this preclinical study, we observed that broad CNS delivery of our vector is possible using an IV route, which is far less invasive than the IT or ICV routes and therefore points to IV as the preferred route of administration in our clinical program for Canavan disease."

Aspa Therapeutics is currently enrolling a natural history study of Canavan disease called CANinform. Interested individuals can find information about the study at **TreatCanavan.com**.

About Adrenas Therapeutics

Adrenas Therapeutics is a BridgeBio Pharma subsidiary developing a gene therapy treatment for congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency, a rare genetic disorder which affects the ability of the adrenal glands to properly function. People with CAH have an impaired ability to produce the hormone cortisol, which can result in life-threatening adrenal crises and significant morbidity throughout life.

About Aspa Therapeutics

Aspa Therapeutics is a BridgeBio Pharma subsidiary focused on developing a gene therapy to treat Canavan disease, an extremely rare genetic disease caused by an inherited mutation of the ASPA gene wherein children affected experience lack of head control, lack of muscle tone (often resulting in floppiness or spasticity) and seizures. Most children are not able to meet developmental milestones, are unable to crawl, walk, sit or talk, and pass away at a young age.

About BridgeBio Pharma

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development. For more information, please visit **www.bridgebio.com**.

BridgeBio Pharma Forward-Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, 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 Securities 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 clinical and therapeutic benefits of BBP-631 and BBP-812, the potential of BBP-631 to be a meaningful therapy for patients with congenital adrenal hyperplasia with 21-hydroxylase deficiency and the potential for BBP-812 to overcome the challenges associated with other gene therapies' inability to target the CNS, Adrenas Therapeutics' and Aspa Therapeutics' clinical development plans, including their plans to file their respective INDs in 2020, and timing and completion of preclinical studies and regulatory submissions, 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 variety of risks and factors that are beyond our control including, without limitation, Adrenas Therapeutics' and Aspa Therapeutics' ability to continue its planned development and regulatory submissions for BBP-631 and BBP-812, respectively, and the timing and success of any such continued clinical development and planned regulatory submissions, as well as those set forth in the Risk Factors section of BridgeBio Pharma Inc.'s most recent Quarterly Report on Form 10-Q and our other SEC filings. Except as required by 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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SOURCE BridgeBio Pharma

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