



NEWS RELEASE

bridgebio pharma grows pipeline to 20+ genetic medicines with four assets focused on treating genetically driven diseases at their source

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-New assets span multiple modalities and target an endocrine disorder, inherited eye disease, genetically driven deafness and autism spectrum disorder

SAN FRANCISCO, Jan. 13, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (NASDAQ: BBIO) today disclosed four additional assets in its pipeline, expanding the number of disclosed programs to over 20 potential medicines. BridgeBio's world-class team of experienced drug developers are advancing these therapies with the aim of delivering life-changing medicines to patients. BridgeBio founder and CEO Neil Kumar, Ph.D., will discuss these new assets and BridgeBio's pipeline during a presentation at the 38th Annual J.P. Morgan Healthcare Conference today at 3 PM (PST) in San Francisco.

The presentation will be webcast live and can be accessed at www.bridgebio.com on the For Investors page under News & Events.

"The range of disorders driven by genetic mutations is vast, and our increasing understanding of the biology of such conditions is allowing us to better engineer potential therapies designed to alleviate genetically driven diseases," said Dr. Kumar, CEO of BridgeBio. "We are excited to unveil these four programs we've had the privilege of working on, taking advantage of research done by companies and academic investigators on the leading edge of scientific discovery. Our teams of experts in cardiovascular and renal disease, gene therapy, and ophthalmology identified these opportunities and have been working to advance them, in some cases for more than a year."

Encaleret is a small molecule antagonist of the calcium sensing receptor targeting conditions related to hypoparathyroidism including Autosomal Dominant Hypocalcemia Type 1 (ADH1). Individuals with ADH1 typically have low serum calcium and high urine calcium due to gain-of-function mutations in the calcium sensing receptor. BridgeBio completed the submission of an Investigational New Drug (IND) application to the US Food and Drug Administration in late 2019 to support initial development of encaleret. A Phase 2b study of encaleret in ADH1 is planned to initiate in early 2020 at the National Institutes of Health (NIH) and expected to provide proof-of-concept data in 2021. Encaleret is housed in BridgeBio subsidiary Calcilytix Therapeutics.

BBP-551 is a novel treatment for the genetically determined retinal diseases phenotypically classified as Retinitis Pigmentosa and Leber's Congenital Amaurosis and genotypically caused by mutations of Retinal Pigment Epithelium Protein 65 (RPE65) or Lecithin:Retinol Acyltransferase (LRAT). Each of these inborn errors of metabolism disrupts key steps within the visual cycle in regenerating 11-cis-retinal, leading to a deficiency in the molecule necessary for rhodopsin formation and subsequent visual signal transduction. Both lead to progressive visual loss and retinal degeneration and, eventually, blindness. The therapy originated in the lab of Dr. Krzysztof Palczewski, Ph.D. and was advanced by David Saperstein, M.D., both at the University of Washington. BBP-551 has been granted Orphan Drug Designation in the United States and European Union and Fast Track Designation in the United States. BBP-551 is housed in BridgeBio subsidiary Retinagenix Therapeutics.

BBP-815 is an AAV gene therapy for the treatment for nonsyndromic hearing loss caused by recessive mutations in the TMC1 gene. TMC1 encodes the mechanosensory ion channel that converts sound vibrations in the inner ear into electrical signals in sensory hair cells. Mutations in the TMC1 gene prevent sound from eliciting the appropriate electrical response in the hair cells, resulting in moderate to severe hearing loss, often present early in life. By replacing the dysfunctional TMC1 protein using AAV gene therapy, BBP-815 is aiming to repair the deficient inner ear sensory cells at the source of this disease. The initial work for this treatment was performed by Jeffrey Holt, Ph.D, Professor of Otolaryngology and Neurology at Boston Children's Hospital and Harvard Medical School. Dr. Holt is a world expert in hearing loss gene therapy and elucidated TMC1's role in hearing. BBP-815 is housed in BridgeBio subsidiary Audition Therapeutics.

BBP-472 is a novel treatment designed to balance kinase signaling in the brain for the treatment of children with autism-spectrum disorders (ASD) characterized by loss of the PTEN protein. This program, currently in the lead-finding phase, is focused on advancing a brain-permeable inhibitor of PI3KB, a kinase shown to signal unabatedly in the absence of PTEN. Although PI3KB inhibitors designed to stay out of the brain are being tested experimentally for cancer indications, a PI3KB inhibitor engineered for brain penetrance has not been advanced to date, representing a major unmet need for this pediatric population.

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 20 development programs includes product candidates ranging from early discovery to late-stage development. For more information, visit bridgebio.com.

BridgeBio Pharma 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 expectations, plans and prospects regarding the preclinical and clinical development plans, clinical trial designs, clinical and therapeutic potential, and strategy of BridgeBio's product candidates, including, but not limited to, encaleret, BBP-551, BBP-815 and BBP-472, BridgeBio's ability to continue to rapidly identify and develop therapeutic innovations, the timing and acceptance of Calcilytix's Investigational New Drug (IND) application for encaleret, BridgeBio's subsidiaries' ability to advance their product candidates through clinical development, the timing and expected results of the Phase 2b study of encaleret in ADH1, BridgeBio's subsidiaries' ability to maintain current and enter into new collaborations with partners, including Calcilytix's partnership with the National Institutes of Health, the ability of BBP-551 to retain Orphan Drug Designation in the United States and European Union and Fast Track Designation in the United States, and the timing of these events, 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, the estimated therapeutic potential of BridgeBio's subsidiaries' product candidates, the timing and acceptance of Calcilytix's IND for encaleret, the timing and success of the Phase 2b study of encaleret in ADH1, Calcilytix's ability to maintain and derive benefits from its partnership with the NIH, and BridgeBio's ability to support its subsidiaries' product candidates and portfolios and advance their product candidates through clinical development, as well as

those risks 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. Moreover, BridgeBio operates 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 BridgeBio's management as of the date of this 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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