



NEWS RELEASE

## bridgebio pharma announces proof-of-concept data of encalaret in adh1 at the endocrine society's 2021 annual meeting

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Figure 1

Baseline and Day 5 blood and urine calcium levels

- Normalization of Blood Calcium and Urine Calcium in 6 of 6 (100%) Autosomal Dominant Hypocalcemia Type 1 (ADH1) Participants Initially Evaluated Over 5 Days Demonstrates Proof-of-Concept
- At Doses up to 180 mg Twice Daily, Encalaret was Well-Tolerated with No Serious Adverse Events and No Adverse Events of Moderate or Severe Intensity Reported After 5 Days
- Company to Host Webcast to Discuss Data on March 22 at 8:00 a.m. ET

PALO ALTO, Calif., March 20, 2021 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO), a clinical-stage biopharmaceutical company founded to discover, create, test and deliver meaningful medicines for patients with genetic diseases and cancers with clear genetic drivers, today announced early results from an ongoing Phase 2b proof-of-concept, open-label study of encalaret for the treatment of Autosomal Dominant Hypocalcemia Type 1 (ADH1). The data are featured in an ePoster presentation titled 'The Effects of Encalaret (CLTX-305) on Mineral Physiology in ADH1 Demonstrate Proof-of-Concept: Early Results from an Ongoing Phase 2B, Open-Label, Dose-Ranging Study' at the Endocrine Society's 2021 Annual Meeting (ENDO 2021) taking place virtually from March 20<sup>th</sup> – 23<sup>rd</sup>.

"ADH1 is a rare genetic form of hypoparathyroidism caused by pathogenic variants in the calcium-sensing receptor

(CASR) gene that are estimated to be harbored by 12,000 individuals in the United States<sup>1</sup>. Patients with ADH1 experience a range of symptoms associated with low blood calcium and high urine calcium, which cannot be adequately addressed with current standard of care therapies,” said Rachel Gafni, M.D., Senior Research Physician and Head, Mineral Homeostasis Studies Group of the National Institute of Dental and Craniofacial Research, National Institutes of Health (NIH). “In these initial results from the Phase 2b study of encalaret in ADH1, blood and urine calcium levels were normalized in all trial participants within five days of treatment.”

In these data from the ongoing Phase 2b open-label, dose-ranging study, six adults with ADH1 with four distinct CASR genotypes were administered encalaret. Calcitriol (active Vitamin D) therapy (current standard of care) was discontinued prior to and throughout the study. Non-dietary calcium supplements were withheld in five participants with adequate dietary calcium intake. Participants received sequential, increasing daily doses of encalaret starting at 30 mg while undergoing intensive safety monitoring and frequent blood and urine sampling for biochemical measures. Following five days of dosing with encalaret, blood calcium, parathyroid hormone, phosphorus and magnesium were within the normal range on average. Urinary calcium excretion was normal or undetectable in all participants.

Baseline and day 5 blood and urine calcium levels are summarized in the figure below:

<https://www.globenewswire.com/NewsRoom/AttachmentNg/09f2eee6-0e25-4c52-aa4b-83674ad51af5>

Throughout this initial period of dose escalation, encalaret was well-tolerated with no serious adverse events and no adverse events of moderate or severe intensity reported. Two participants experienced transient, asymptomatic hypophosphatemia which was the only treatment-related adverse event. The tolerability and consistent mineral responses following encalaret administration in all six ADH1 trial participants demonstrates proof of concept that encalaret may be an efficacious therapy option for ADH1.

The Company intends to meet with regulatory health authorities in 2021 to discuss potential paths to registration prior to initiation of a Phase 3 registrational study in patients with ADH1. If the development program is successful, encalaret could be the first approved therapy option indicated specifically for the treatment of ADH1.

“A close collaboration between world-leading experts in calcium homeostasis at the National Institute of Dental and Craniofacial Research at the NIH and BridgeBio has made possible the development of encalaret for ADH1,” said Jonathan Fox, M.D., Ph.D., Chief Medical Officer of Calcilytix, an affiliate of BridgeBio that is focused on developing encalaret. “We look forward to working with regulators this year to define a potential path to approval for encalaret for the treatment of ADH1 and to exploring its potential use in patients with other forms of hypoparathyroidism.”

At ENDO 2021, BridgeBio also presented clinical study designs for the Phase 2b study of encalaret in ADH1; for its

Phase 2 study of low-dose infigratinib, an FGFR1-3 inhibitor, for children with achondroplasia, the most common form of genetic short stature; and for its Phase 1/2 study of its investigational AAV5 gene therapy candidate for congenital adrenal hyperplasia (CAH). CAH is one of the most prevalent genetic diseases that could potentially be addressable with AAV gene therapy.

<sup>1</sup> Dershem et al., Amer Jour of Hum Genetics, 2020

#### Webcast Information

BridgeBio will host a conference call and simultaneous webcast to share updates on the encalceret proof-of-concept data in ADH1 on March 22 at 8:00 a.m. ET. To access this call, dial (800) 379-2666 (U.S.) or (409) 937-8964 (International). Conference ID: 7371879. A link to the webcast may be accessed from the event calendar page of BridgeBio's website at <https://investor.bridgebio.com/>. A replay of the conference call and webcast will be archived on the Company's website and will be available for at least 30 days following the event.

#### About BridgeBio Pharma, Inc.

BridgeBio is a 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 over 30 development programs ranges from early science to advanced clinical trials and its commercial organization is focused on delivering the company's first approved therapy. 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](https://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 our product candidates, including, but not limited to: the unknown future impact of the COVID-19 pandemic delay on our ongoing clinical trials and/or our operations or operating expenses; early results from our ongoing Phase 2b proof-of-concept, open-label study of encalceret for the treatment of Autosomal Dominant Hypocalcemia Type 1 (ADH1) being

indicative of final data from our Phase 2b study of encalaret; the potential size of the target patient population with a rare genetic form of hypoparathyroidism caused by pathogenic variants in the calcium-sensing receptor (CASR) gene; the inability of current standard of care therapies to treat ADH1; encalaret continuing to be well-tolerated with no serious adverse events and no adverse events of moderate or severe intensity reported in our ongoing Phase 2b proof-of concept, open-label study; tolerability and consistent mineral responses following encalaret administration in all six ADH1 trial participants continuing to demonstrate proof-of-concept that encalaret may be an efficacious therapy option for ADH1; the timing and success of our planned meetings with regulatory health authorities, including the U.S. Food and Drug Administration (FDA), in 2021 to discuss potential paths to registration prior to initiation of a Phase 3 registrational study in patients with ADH1; the ability of encalaret to be the first approved therapy option indicated specifically for the treatment of ADH1, if the development program is successful; the continuing close collaboration between world-leading experts in calcium homeostasis at the National Institute of Dental and Craniofacial Research at the National Institutes of Health and BridgeBio; the exploration of encalaret's potential use in patients with other forms of hypoparathyroidism; the clinical study designs for our Phase 2b study of encalaret in ADH1, for our Phase 2 study of low-dose infigratinib, an FGFR1-3 inhibitor for children with achondroplasia, and for our Phase 1/2 study of our investigational AAV5 gene therapy candidate for Congenital Adrenal Hyperplasia (CAH); CAH being one of the most prevalent genetic diseases that could potentially be addressable with AAV gene therapy;; 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: early data from our ongoing Phase 2b proof-of-concept, open-label study of encalaret for the treatment of ADH1 not being indicative of final data; the potential size of the target patient population for ADH1 not being as large as anticipated; encalaret not being well-tolerated, with serious adverse events and adverse events of moderate or severe intensity being reported in the final Phase 2b study data; encalaret not continuing to demonstrate that it may be an efficacious therapy option for ADH1 based on the final Phase 2b data; encalaret not being the first approved therapy option indicated specifically for the treatment of ADH1, if the development program is not successful or if a competing therapy option is approved; 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 prior to initiation of a Phase 3 registrational study of encalaret in patients with ADH1, the FDA or such other regulatory agencies may not agree with our regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted; the continuing success of our close collaboration between the National Institute of Dental and Craniofacial Research at the National Institutes of Health; the inability of encalaret to be used in patients with other forms of

hypoparathyroidism; potential adverse impacts due to the global COVID-19 pandemic such as delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy; and those risks set forth in the Risk Factors section of our most recent quarterly or annual periodic report filed with the U.S. Securities and Exchange Commission (SEC) 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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