BridgeBio Pharma Receives FDA Fast Track Designation for Encaleret for the Treatment of Autosomal Dominant Hypocalcemia Type 1

PALO ALTO, CA – June 1, 2021 — BridgeBio Pharma, Inc. (Nasdaq: BBIO), a commercial-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 that the U.S. Food and Drug Administration (FDA) granted Fast Track designation for encaleret for the treatment of autosomal dominant hypocalcemia (ADH1). The FDA Fast Track designation program is designed to facilitate the development and to expedite the review of new therapies hoping to treat or prevent serious conditions and fill an unmet medical need. BridgeBio announced the Fast Track designation on World Hypoparathyroidism Awareness Day, an annual global awareness and education event designed to spotlight and support people living with ADH1 and other types of hypoparathyroidism.

ADH1 is a rare, genetic form of hypoparathyroidism caused by pathogenic variants in the calcium-sensing receptor gene (*CASR*). It is estimated that about 12,000 individuals in the United States carry such variants in *CASR.*[1] The calcium-sensing receptor gene encodes the receptor, CaSR, which senses the level of calcium in the body and regulates the amount of calcium in the blood through its effects on the parathyroid glands, the kidney, and bone. Gain-of-function variants in *CASR* result in sensing low calcium as normal. As a result, patients with ADH1 have low blood calcium (hypocalcemia), low or low-normal parathyroid hormone levels, and excess urinary excretion of calcium (hypercalciuria). Hypocalcemia can cause severe muscle cramping and seizures, while hypercalciuria can lead to impaired kidney function and kidney stone formation. The current standard-of-care for ADH1 patients consists of oral calcium supplements in excess of typical dietary requirements for people with normal CaSR function, and activated vitamin D, which can partially correct hypocalcemia but typically worsens both hypoparathyroidism and hypercalciuria.

“Balancing near-normal blood and avoiding excess urinary calcium is a daily struggle for patients with ADH1 as the range of symptoms produced by the highs and lows of the condition cannot adequately be addressed by current standard-of-care,” said Jonathan Fox, M.D., Ph.D., Chief Medical Officer of the cardio-renal affiliates at BridgeBio, including Calcilytix, which is focused on developing encaleret. “With respect to the mechanism of disease, which is driven by gain-of-function variants in *CASR*, encaleret, as an allosteric negative modulator of the receptor’s calcium sensing activity, has the potential to correct the disease mechanism at its source. It is encouraging to receive Fast Track designation from the FDA as it recognizes the seriousness of ADH1 and the potential for encaleret to address this profound unmet medical need.”

Promising early results from its ongoing Phase 2b proof-of-concept, open-label study of encaleret, orally administered, for patients with ADH1 were presented at the Endocrine Society’s 2021 Annual Meeting, which showed the normalization of blood calcium and urine calcium in six of six (100%) ADH1 participants evaluated over five days and demonstrated clinical proof-of-concept.[2] BridgeBio plans to engage with regulatory health authorities to

discuss the design of a Phase 3 registrational study in patients with ADH1. If the development program is successful, encaleret could be the first approved therapy indicated specifically for the treatment of ADH1.

For more information on the Phase 2b clinical trial currently recruiting participants with ADH1, please visit clinicaltrials.gov (Identifier: NCT04581629).

[1] Dershem et al., Amer Jour of Hum Genetics, 2020.
[2] Gafni et al., Jour of Endo Soc, 2021.

About BridgeBio Pharma, Inc.
BridgeBio Pharma (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**.

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: early results from our ongoing Phase 2b proof-of-concept, open-label study of encaleret for the treatment of Autosomal Dominant Hypocalcemia Type 1 (ADH1) being indicative of final data from our Phase 2b study of encaleret; the potential size of the target patient population for ADH1; the inability of current standard-of-care therapies to treat ADH1; the timing and success of our planned meetings with regulatory health authorities, including the U.S. Food and Drug Administration (FDA), in 2021, including regarding the design of a Phase 3 registrational study in patients with ADH1; the ability of encaleret to be the first approved therapy option indicated specifically for the treatment of ADH1, if the development program is successful; the unknown future impact of the COVID-19 pandemic delay on our ongoing clinical trials and/or our operations or operating expenses; 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 encaleret for the treatment of ADH1 not being indicative of final data; encaleret 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; 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 encaleret 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; 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.

###

BridgeBio Contact:
Grace Rauh
BridgeBio Pharma, Inc.
Grace.rauh@bridgebio.com
(917) 232-54

Patient Advocacy Contact:
Jocelyn Ashford
BridgeBio Pharma, Inc.
jocelyn.ashford@bridgebio.com
(650) 452-4199

Source: BridgeBio Pharma, Inc.