NEWS RELEASE

bridgebio pharma announces positive feedback from the u.s. fda and eu ema on the regulatory path for a pivotal phase 3 trial of infigratinib in children with achondroplasia

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- BridgeBio completed an end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA), and a scientific advice engagement with the European Union (EU) European Medicines Agency (EMA)

- FDA and EMA alignment was reached on the adequacy of a one-year, 2:1 randomized, placebo-controlled Phase 3 pivotal trial for infigratinib to support a marketing application for the treatment of children with achondroplasia

- The primary endpoint will be change from baseline in annualized height velocity (AHV) at 12 months, with secondary endpoints including proportionality, height Z-score and impact on medical complications

- These regulatory interactions clear the path for the initiation of PROPEL3 by the end of 2023

- BridgeBio had previously announced a +3.38cm/yr increase from baseline in annualized height velocity (AHV) at six months at 0.25mg/kg/day, with no serious adverse events (SAEs) or adverse events (AEs) assessed as related to study drug

PALO ALTO, Calif., Sept. 06, 2023 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today announced positive feedback from the U.S. FDA and the EU EMA on key elements of the trial design for PROPEL3, its pivotal Phase 3

study of infigratinib in children with achondroplasia. Both regulatory bodies indicated that a single, one-year, 2:1 randomized, placebo-controlled trial would be acceptable as a registrational study for approval, which clears the path for initiation of the trial by the end of 2023.

”The regulatory feedback clearing the path to approval for infigratinib is great news for children living with achondroplasia and for physicians who treat them. It is incredibly exciting that infigratinib has the potential to become the first effective oral treatment option to improve growth, enhance functionality and decrease complications,” said Dr. Melita Irving, M.D., a clinical geneticist at Guy’s and St Thomas’ NHS Foundation Trust, London, UK and investigator for the infigratinib clinical program at the Evelina London Children’s Hospital.

PROPEL3, the Phase 3 registrational study is a randomized, double-blinded, multi-site, placebo-controlled clinical trial, which will evaluate the safety and efficacy of infigratinib in children with achondroplasia aged 3 to 18 years with open growth plates. The primary endpoint will be change from baseline in AHV, with secondary endpoints including proportionality, height Z-score, and impact on medical complications. BridgeBio is also committed to exploring the potential of infigratinib on the wider medical and functional impacts of achondroplasia, hypochondroplasia and other skeletal dysplasias, which hold significant unmet needs for families. Based on the positive results to date, the Company has been enrolling children in the run-in for PROPEL3 and expects to initiate PROPEL3 by the end of the year.

Cohort 5 of PROPEL2, BridgeBio’s Phase 2 dose-finding study, demonstrated that the dose level of 0.25 mg/kg/day resulted in a significant and robust increase in AHV, with a mean change of +3.38 cm/year from baseline at 6 months. The findings also highlighted early but promising trends towards improvement in proportionality, as measured by the upper and lower body segment ratio study. Additionally, the study has shown a well-tolerated safety profile, with no study drug related treatment emergent adverse events, SAEs or discontinuations due to AEs at the selected dose.

”We are thrilled to have regulatory alignment on key elements of our Phase 3 trial design and the path towards submitting a marketing application to both the FDA and the EMA. We are appreciative of the support from both agencies and plan to move forward swiftly with initiation and dosing in the pivotal trial, with the hopes of providing a much needed oral option to children with achondroplasia,” said Adora Ndu, Pharm.D., J.D., chief regulatory officer of BridgeBio.

Additionally, BridgeBio expects to initiate clinical development for infigratinib in hypochondroplasia, a skeletal dysplasia closely related to achondroplasia and similarly driven by FGFR3 gain-of-function variants. BridgeBio has previously presented promising preclinical data for hypochondroplasia at ENDO 2023 and ASHG 2022.

About Achondroplasia

Achondroplasia is the most common cause of disproportionate short stature, affecting approximately 55,000 people in the U.S. and EU, including up to 10,000 children and adolescents with open growth plates. Achondroplasia impacts overall health and quality of life, leading to medical complications such as obstructive sleep apnea, middle ear dysfunction, kyphosis, and spinal stenosis. The condition is uniformly caused by an activating mutation in FGFR3.

About BridgeBio Pharma, Inc.

BridgeBio Pharma (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 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. These forward-looking statements, including statements relating to the clinical, therapeutic and market potential of our programs and product candidates, including our clinical development program for infigratinib in achondroplasia, the timing and success of our clinical development programs, the progress of our ongoing and planned clinical trials of infigratinib in achondroplasia and in hypochondroplasia, including the status of enrollment and the expectation of initiating a Phase 3 trial for infigratinib in achondroplasia (PROPEL3) by the end of 2023, the design of PROPEL3, including the expectation that PROPEL3 will evaluate the safety and efficacy of infigratinib in children with achondroplasia aged 3 to 18 years with open growth plates, the primary endpoint and the secondary endpoints of such trial; the expectations of our interactions with regulatory authorities, including the path towards submitting a marketing application to both the FDA and the EMA after our regulatory alignments on the key elements of the PROPEL3 design, the statements regarding such path in the quotes of Dr. Irving and Dr. Ndu; the potential benefits of infigratinib, including the statement of the benefit of infigratinib in Dr. Irving's quote; our exploration of the potential of infigratinib on the wider medical and functional impacts of achondroplasia, hypochondroplasia and other skeletal dysplasias, and the expectation to initiate clinical development in hypochondroplasia, the availability of data from our clinical trials of infigratinib, and the timing of these events,

reflect our current views about our plans, intentions, expectations and strategies, 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, initial and ongoing data from our clinical trials not being indicative of final data, the design and success of ongoing and planned clinical trials, difficulties with enrollment in our clinical trials, adverse events that may be encountered in our clinical trials, the FDA or 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, 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, the impacts of current macroeconomic and geopolitical events, including changing conditions from the COVID-19 pandemic, hostilities in Ukraine, increasing rates of inflation and rising interest rates, on our overall 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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