



NEWS RELEASE

bridgebio announces first child dosed in propel 3, its phase 3 clinical trial for infigratinib in children with achondroplasia

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- BridgeBio has dosed the first child in PROPEL 3, a one-year, 2:1 randomized, placebo-controlled Phase 3 pivotal trial evaluating the efficacy and safety of infigratinib in children with achondroplasia
- The U.S. Food and Drug Administration (FDA) and European Union (EU) European Medicines Agency (EMA) shared positive feedback that the PROPEL 3 trial design would be acceptable as a registrational study to support a marketing application
- Data from PROPEL 2, BridgeBio's Phase 2 clinical trial of infigratinib in children with achondroplasia, supports infigratinib's potential as an oral, well-tolerated treatment option; results shared at ENDO 2023 included a significant increase in annual height velocity (AHV) with a mean change of +3.38 cm/year from baseline at six months, and early but promising trends towards improvement in proportionality, as measured by the upper and lower body segment ratio
- BridgeBio expects to begin development of infigratinib in hypochondroplasia, with the initiation of an observational lead-in study in the first half of 2024

PALO ALTO, Calif., Dec. 13, 2023 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, today announced that the first child has been dosed in PROPEL 3, a Phase 3 clinical trial studying the efficacy and safety of infigratinib in children with achondroplasia. Both the U.S. FDA and the EU EMA indicated the trial design for PROPEL 3 would be

acceptable as a registrational study to support a marketing application for the treatment of children with achondroplasia.

“The Phase 2 data for infigratinib has been very promising and suggests potential to increase growth, improve functionality and reduce the associated medical complications in children with achondroplasia. With the recent dosing of the first child in the Phase 3 trial, I am hopeful that we are one step closer to providing a safe, effective oral therapy to the people in the achondroplasia community who are seeking treatment,” said Dr. Ravi Savarirayan, M.D., Ph.D., clinical geneticist and leader of the molecular therapies research group at the Murdoch Children’s Research Institute in Melbourne, Australia and the global lead investigator for PROPEL 3.

PROPEL 3 is a global, one-year, 2:1 randomized, double-blinded placebo-controlled clinical trial, which will evaluate the efficacy and safety 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 (a measure of variance and dispersion from the average height of children with achondroplasia), and impact on medical complications and quality of life.

“We are thrilled about this major milestone for our program and are hopeful about what this means for those families seeking a therapeutic option. We are grateful to the children and their families for participating in our Phase 3 trial as well as the open collaboration we have built with physicians and advocacy groups to best understand the needs of those living with this condition. We look forward to continuing to explore the benefits of infigratinib not only in growth, but in the functional improvements and the alleviation of medical complications that are most meaningful to the community,” said Daniela Rogoff, M.D., Ph.D., senior vice president of clinical development for skeletal dysplasias at BridgeBio.

In June 2023, the Company presented updated six-month results from Cohort 5 of PROPEL 2, its Phase 2 dose-finding study, at ENDO 2023. Cohort 5 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 six months. The findings also highlighted early but promising trends towards improvement in proportionality, as measured by the upper and lower body segment ratio. Additionally, the results showed a well-tolerated safety profile, with no study drug related treatment emergent adverse events, serious adverse events or discontinuations due to adverse events at the 0.25 mg/kg dose.

“Achondroplasia can impact the overall health and wellbeing of a person, including functional limitations, social stigma and medical complications. We appreciate the opportunity to work with BridgeBio to ensure the needs of our community are heard, and we are excited by what the Phase 3 study could bring for those looking for options to help themselves or their loved ones,” said Susana Noval, director of Fundación ALPE Acondroplasia. Fundación

ALPE Acondroplasia, based in Gijón, Spain, is an advocacy organization for people with achondroplasia and other skeletal dysplasias and their families.

Information about PROPEL 3 (NCT06164951) can be found **here** on clinicaltrials.gov. Information about PROPEL (NCT04035811), BridgeBio's observational lead-in study in achondroplasia for PROPEL 3 and other studies, can be found **here** on clinicaltrials.gov. Additionally, BridgeBio expects to initiate ACCEL, an observational lead-in study for infigratinib in hypochondroplasia, a skeletal dysplasia closely related to achondroplasia and similarly driven by FGFR3 gain-of-function variants, in the first half of 2024. BridgeBio has previously presented promising preclinical data for hypochondroplasia at ENDO 2023 and ASHG 2022. BridgeBio is committed to exploring the potential of infigratinib on wider medical and functional impacts of achondroplasia, hypochondroplasia and other skeletal dysplasias, which hold significant unmet needs for families.

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 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 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 indications by both the U.S. FDA and the EU EMA that the trial design for PROPEL 3 would be acceptable as a registrational study to support a marketing application for the treatment of children with achondroplasia; the statements regarding the potential benefits of infigratinib and the benefits of the Phase 3 study, including such statements in the quotes of Dr. Savarirayan, Dr. Rogoff and Ms. Noval; our exploration of the potential of infigratinib on wider medical and functional impacts of achondroplasia, hypochondroplasia and other skeletal dysplasias; and the expectation to begin development of infigratinib in hypochondroplasia, with the initiation of a observational lead-in study in the first half of 2024, 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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