



NEWS RELEASE

## bridgebio pharma presents updated six month results from its phase 2 cohort 5 trial of infigratinib in achondroplasia at the endocrine society (endo) 2023 annual conference

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- Treatment with infigratinib at the Cohort 5 dose level resulted in a significant and robust increase in annual height velocity (AHV), with a mean change of +3.38 cm/year from baseline
- 83% of children in Cohort 5 responded to infigratinib, as defined by an increase from baseline AHV of at least 25%. The mean change from baseline in AHV of responders was +4.08 cm/year
- Early but promising trends towards improvement in proportionality were observed, as measured by the upper and lower body segment ratio
- At six months, infigratinib was well-tolerated as a single daily oral therapy with no adverse events (AEs) assessed as treatment-related in all patients in Cohort 5
- Based on the positive Phase 2 results, BridgeBio is currently enrolling children for a pivotal Phase 3 trial

PALO ALTO, Calif., June 20, 2023 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) ("BridgeBio" or the "Company"), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, presented updated six month results from Cohort 5 in PROPEL2, a Phase 2 trial of the investigational therapy infigratinib in children with achondroplasia, demonstrating a continued potentially best-in-class efficacy and safety profile at the

Endocrine Society (ENDO) 2023 Annual Conference. Infigratinib is an oral small molecule designed to inhibit FGFR3 and target achondroplasia at its source.

“The safety and efficacy results seen in these Phase 2 data are extremely promising and suggest that infigratinib has the potential to be the first effective oral therapy to improve growth, enhance functionality and decrease complications in children with achondroplasia. We consider that the cumulative increases in growth velocity will translate to improvements for the medical and functional complications of achondroplasia, which will be impactful for those seeking treatment within the community,” 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 PROPEL2.

The updated six month results for 12 patients in Cohort 5 (0.25mg/kg/once daily) include:

- The mean increase from baseline in AHV is significant and robust at +3.38 cm/year
- 83% of the children were responders, based on the criteria defined for the study with a change from baseline AHV of at least 25%, with a mean of +4.08 cm/year
- Preliminary data suggests the Cohort 5 dose level may be having a positive effect on the upper and lower body segment ratio
- Changes in linear growth are supported by an increase in collagen X marker (CXM), an independent, real-time biomarker of bone growth, supporting a true biologic effect from infigratinib
- This increase in growth translated into an increase in z-score of +0.29 standard deviation scores compared to achondroplasia growth charts
- Treatment has been well tolerated, with no serious adverse events (SAEs), or treatment emergent AEs that led to treatment discontinuation

In addition to the Cohort 5 data being shared, the Phase 2 data from the previous four cohorts was also discussed during the presentation. Treatment in all cohorts has been well tolerated with no SAEs or any AEs requiring treatment discontinuation. The AEs most frequently reported are considered common conditions in the pediatric population, particularly in children with achondroplasia. The Phase 2 trial was designed to identify the dose of infigratinib that will be explored in the Phase 3 trial.

“I am encouraged by the consistent results we are continuing to see in AHV. Our data also presents the opportunity for us to move forward quickly in initiating a Phase 3 clinical trial. We appreciate the ongoing support and partnership by the physicians, community advocates, children, and families in this study and look forward to continuing to explore the benefits of infigratinib not only in growth, but in other outcomes meaningful to the community,” said Daniela Rogoff, M.D., Ph.D., senior vice president of clinical development for the skeletal dysplasias program at BridgeBio.

Based on the positive results to date, BridgeBio is underway in enrolling children in the run-in for a Phase 3 trial. For more information, please visit [QEDtx.com](https://www.qedtx.com). Additionally, the Company expects to initiate clinical development for infigratinib in hypochondroplasia, a skeletal dysplasia related to achondroplasia and similarly driven by FGFR3 gain-of-function variants, in the coming months. Infigratinib has IP protection out to at least 2041.

At ENDO 2023, BridgeBio also shared nonclinical results from studies in skeletal dysplasias as well as updates from its autosomal dominant hypocalcemia type 1 (ADH1) program, including positive 18 month data from its ongoing Phase 2 study of encaleret in ADH1 and its sponsored hypoparathyroidism genetic testing program.

#### About Achondroplasia

Achondroplasia is the most common cause of disproportionate short stature, affecting approximately 55,000 people in the United States (US) and European Union (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 Inc. (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](https://www.bridgebio.com) and follow us on [LinkedIn](#) and [Twitter](#).

#### BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements BridgeBio makes 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. BridgeBio intends 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, our clinical development program for infigratinib in achondroplasia, including the potential of infigratinib to be the first effective oral therapy to improve growth, enhance functionality and decrease complications in children with 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 our plan to initiate a Phase 3 trial for infigratinib in achondroplasia and the plan and the timing to initiate clinical development in hypochondroplasia, our planned interactions with regulatory authorities, 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 COVID-19, 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 hostilities in Ukraine, increasing rates of inflation and rising interest rates, on 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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