



NEWS RELEASE

bridgebio announces durable month 12 and 18 phase 2 cohort 5 results of oral infigratinib in achondroplasia, and first participant consented in accel for hypochondroplasia

2024-06-04

- In Cohort 5 of PROPEL 2 (0.25 mg/kg/day), oral treatment with infigratinib resulted in a statistically significant and sustained increase in annualized height velocity (AHV), with a mean change from baseline of +2.51cm/yr at Month 12, and +2.50 cm/yr at Month 18 (p=0.0015)
- At Month 18, there was a statistically significant improvement in body proportionality (p-value of 0.001). The mean upper to lower body segment ratio was 1.88 at Month 18, as compared to 2.02 at baseline
- Infigratinib continues to be well-tolerated as a single daily oral therapy with no adverse events (AEs) assessed as treatment-related in any participant in Cohort 5
 - PROPEL 3, the global Phase 3 registrational study of infigratinib in achondroplasia, continues to enroll on schedule, with completion estimated by end of 2024
 - BridgeBio announces first child consented in ACCEL, the observational run-in study for infigratinib in children living with hypochondroplasia, a skeletal dysplasia closely related to achondroplasia and similarly driven by FGFR3 gain-of-function variants

PALO ALTO, Calif., June 04, 2024 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio), a

commercial-stage biopharmaceutical company focused on genetic diseases, today announced sustained positive results from PROPEL 2, a Phase 2 trial of the investigational therapy infigratinib in children with achondroplasia, demonstrating continued potential best-in-class efficacy and an encouraging safety profile. Infigratinib is an oral small molecule designed to inhibit FGFR3 signaling and target achondroplasia and hypochondroplasia at their source. BridgeBio will also host an **investor call** on June 4, 2024, at 8:00 am ET with Ravi Savarirayan, M.D., Ph.D., of Murdoch Children's Research Institute in Melbourne, Australia, and the global lead investigator for PROPEL 2, to discuss the results from the Phase 2 study.

To date, key results from the Cohort 5 dose escalation cohort in PROPEL 2 trial include:

- Sustained and statistically significant mean increase in AHV of +2.51cm/year from baseline at 12 months, and +2.50 cm/yr at 18 months (p=0.0015)
- Statistically significant improvement in body proportionality (mean upper to lower body segment ratio), from 2.02 at baseline to 1.88 at Month 18 (mean change from baseline, p=0.001)
- A continued well-tolerated safety profile, with no treatment-related adverse events assessed as related to infigratinib

"These data indicate that treatment with infigratinib is continuing to show increased growth velocity and improvements in body proportionality in children with achondroplasia. This is encouraging and suggests that infigratinib has the potential to enhance functionality for people living with achondroplasia in addition to increasing growth. We hope to see these improvements reflected in the ongoing PROPEL 3 pivotal study that will build toward providing a safe and effective oral therapy to those in the achondroplasia community who are seeking treatment," said Dr. Savarirayan.

PROPEL 3, the global Phase 3 registrational study of infigratinib in achondroplasia, continues to enroll on schedule, with completion of enrollment anticipated by the end of the year.

Given the promising results from PROPEL 2, BridgeBio is committed to expanding the FGFR3-related skeletal dysplasias franchise for infigratinib by accelerating development in hypochondroplasia. Positive interactions with both the U.S. FDA and EMA support development in children with hypochondroplasia, with a small open-label Phase 2 portion testing a single dose of 0.25 mg/kg/day, leading into a double-blinded, placebo-controlled Phase 3 study. ACCEL, the observational lead-in program for hypochondroplasia, was initiated with the first participant consented in May 2024. The interventional program, ACCEL 2/3, will be a global Phase 2/3 multicenter, single-dose study, to evaluate the efficacy and safety of 0.25mg/kg/day of infigratinib in children living with hypochondroplasia. The open-label Phase 2 portion in children aged 5 to 11 years old will be followed by a pivotal Phase 3, one-year, 2:1 randomized, double-blinded, placebo-controlled study in children aged 3 to < 18 years old with growth potential. In addition to changes from baseline in AHV measurements, the study will evaluate changes in other indicators of

growth, body proportions, medical complications associated with hypochondroplasia, and changes in quality-of-life measures. BridgeBio has previously presented promising preclinical data for hypochondroplasia at ENDO 2023 and ASHG 2022.

“We are very excited to see a persistence of response to infigratinib in linear growth. We are especially encouraged by the promising effect on body proportions, which supports infigratinib’s potential to provide benefits that could impact the lives of children with achondroplasia. These results motivate us to continue evaluating infigratinib in other FGFR-related skeletal dysplasias and genetic conditions. The initiation of our observational study in hypochondroplasia and the obtainment of FDA and EMA alignment on the interventional study underlie our excitement for the potential of infigratinib as a treatment option for children with hypochondroplasia,” said Daniela Rogoff, M.D., Ph.D., Chief Medical Officer, Skeletal Dysplasias at BridgeBio.

“The journey of living with skeletal dysplasia varies from person to person, but many are impacted by functional limitations, social stigma and medical complications due to their condition and the way their bones develop. We are encouraged to see infigratinib’s potential to improve body proportionality, which could help address functional complications meaningful to people living with skeletal dysplasia. The Chandler Project values the collaborative partnership we’ve developed with BridgeBio and QED, to ensure that the community’s true needs are prioritized throughout the discovery and development process. We are also thrilled for the launch of an observational study in hypochondroplasia, a community that has been eager for further research and development of treatment options,” said Chandler Crews, founder of The Chandler Project, a patient advocacy organization based in Baltimore, MD.

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. Information about ACCEL (NCT06410976), BridgeBio’s observational lead-in study in hypochondroplasia can be found **here** on clinicaltrials.gov. 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.

Webcast Information

BridgeBio will host an investor call and simultaneous webcast to discuss the Phase 2 data at Months 12 and 18 of infigratinib in children with achondroplasia on June 4, 2024 at 8:00 am ET. 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 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 variant in FGFR3.

About Hypochondroplasia

Hypochondroplasia is also an FGFR3-associated skeletal dysplasia and is a rare condition with similar prevalence in achondroplasia. Hypochondroplasia presents with a wide spectrum of phenotypes including disproportionate short stature, mild joint laxity and macrocephaly. Currently, no treatments for hypochondroplasia are approved in the United States.

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. 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 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," "continues," "estimates," "expects," "hopes," "intends," "may," "plans," "projects," "remains," "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, 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 global health emergencies, including 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 and in Israel and the Gaza Strip, increasing rates of inflation and rising interest rates, on our business operations and expectations, as well as those risks set forth in the Risk Factors section of our most recent Annual Report on Form 10-K 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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