



## NEWS RELEASE

# bridgebio pharma's qed therapeutics doses first child in phase 2 clinical trial of the investigational medicine infigratinib in achondroplasia

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SAN FRANCISCO, July 15, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) affiliate QED Therapeutics announced today that the first child with achondroplasia has been dosed with the investigational medicine infigratinib, an orally available small molecule, that targets the overactivity of fibroblast growth factor receptor 3 (FGFR3) in the PROPEL 2 Phase 2 [clinical trial](#). Achondroplasia is the most common cause of disproportionate short stature.<sup>1</sup>

"With preclinical evidence showing increased growth in the long bones, spine and cranial bones, including the base of the skull, there is the potential for infigratinib to help children with achondroplasia by decreasing the impact of serious medical complications," said Professor Ravi Savarirayan, M.B., B.S., M.D., Ph.D., clinical geneticist and group leader of skeletal biology and disease at Murdoch Children's Research Institute in Australia and the lead investigator for the PROPEL 2 trial. "Additionally, infigratinib is being studied as a once-daily dose taken orally, which is an important factor for administration of therapies to children with achondroplasia."

Achondroplasia is caused by an alteration in the FGFR3 gene, which causes the FGFR3 protein to be overly active.<sup>1</sup> This interferes with skeletal development and can lead to disturbances in bone growth.<sup>1</sup> Infigratinib is an oral investigational medicine that is designed to reduce the activity of FGFR3.

"The start of this clinical trial is the culmination of more than two years of work – first to secure rights to develop infigratinib, which we pursued following the publication of data relating to its potential in achondroplasia, and then

to establish preclinical data showing the efficacy and safety of very low doses of the molecule,” said Michael Henderson, M.D., CEO of QED Therapeutics. “Infigratinib illustrates the heart of what BridgeBio set out to do: leverage the highest quality science to identify and develop potential therapies that target genetically driven conditions at their source. Our hope is that a daily, oral dose of infigratinib, which directly targets FGFR3, can provide health benefits for children with achondroplasia.”

The PROPEL 2 trial is a Phase 2 dose escalation and dose expansion trial and the first clinical trial to study infigratinib at low doses in children with achondroplasia. The goal of the study is to assess safety and measure changes from baseline in annualized height velocity and changes in other health factors. To be eligible for the trial, children must first complete a six-month period of assessment in the **PROPEL** prospective clinical assessment study.

#### About QED Therapeutics

**QED Therapeutics**, an affiliate of BridgeBio Pharma, is a biotechnology company focused on precision medicine for FGFR-driven diseases. Our lead investigational candidate is infigratinib (BGJ398), an orally administered, FGFR1-3 selective tyrosine kinase inhibitor that we are evaluating in clinical studies for the treatment of achondroplasia. We plan to conduct further clinical trials to evaluate the potential for infigratinib to treat patients with FGFR-driven tumor types and rare disorders. At much higher doses, infigratinib has shown activity that we believe to be meaningful in clinical measures, such as overall response rate, in patients with chemotherapy-refractory cholangiocarcinoma with FGFR2 fusions and advanced urothelial carcinoma with FGFR3 genomic alterations. QED intends to submit a New Drug Application (NDA) with the United States Food and Drug Administration (FDA) for second and later-line cholangiocarcinoma in 2020.

For more information on QED Therapeutics, please visit the company’s website at **[www.qedtx.com](http://www.qedtx.com)**.

#### About BridgeBio Pharma, Inc.

**BridgeBio** is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio’s pipeline of over 20 development programs includes product candidates ranging from early discovery to late-stage development. For more information, visit **[bridgebio.com](http://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 the timing, expectations, plans, and potential success of QED Therapeutics’ Phase 2 PROPEL 2 clinical trial, the regulatory approval process, clinical trial designs, clinical development plans, clinical trial results, timing and completion of the PROPEL 2 and other clinical trials, clinical and therapeutic potential of infigratinib for the treatment of achondroplasia or other FGFR-driven diseases, 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, QED Therapeutics’ ability to continue or complete its Phase 2 PROPEL 2 clinical trial, ongoing and planned clinical trials of infigratinib for the potential treatment of achondroplasia and other FGFR-driven, the availability of data from these trials, past data from preclinical and earlier clinical studies not being indicative of future data from clinical trials, its ability to advance infigratinib in clinical development according to its plans, and the timing of these events, as well as those risks set forth in the Risk Factors section of BridgeBio Pharma, Inc.’s most recent Quarterly Report on Form 10-Q and our other SEC filings. Moreover, QED Therapeutics operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. 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.

1. National Institutes of Health. <https://ghr.nlm.nih.gov/condition/achondroplasia> Accessed June 23, 2020.

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