



NEWS RELEASE

bridgebio pharma's qed therapeutics announces preclinical data demonstrating potential of low-dose infigratinib in achondroplasia

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Data Accepted to ENDO 2020

SAN FRANCISCO, May 11, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) affiliate QED Therapeutics announced today that in vitro and in vivo data from two studies support QED's plans to evaluate a low dose of infigratinib as a treatment option for children with achondroplasia, the most common cause of disproportionate short stature. Data were accepted for presentation at the Endocrine Society's Annual Meeting ("ENDO 2020"), which was cancelled due to COVID-19, and the studies were published in the special supplemental section of the Journal of the Endocrine Society.

The first study, entitled "**Support for a new therapeutic approach of using a low-dose FGFR tyrosine kinase inhibitor (infigratinib) for achondroplasia.**" showed that in a mouse model of achondroplasia, treatment with infigratinib led to dose-dependent improvement in achondroplasia-associated phenotypes. At a low dose of 0.5 mg/kg, infigratinib was associated with a statistically significant improvement in bone length of 7% to 14% in the upper limbs, 10% to 17% in the lower limbs and 12% in the foramen magnum (the opening at the base of the skull). Also presented in this study was in vitro data demonstrating that, at similar concentrations, infigratinib had greater activity over a CNP analog (vosoritide) in an achondroplasia cell line. The data suggest that inhibition of multiple key pathways downstream of FGFR3 controlling either proliferation or differentiation of the chondrocytes may lead to better efficacy compared with MAPK inhibition alone.

The second study, entitled "**Low-dose infigratinib treatment does not lead to changes in phosphorus in**



preclinical animal studies,” showed that rats and mice treated with infigratinib at or below 5 mg/kg showed no relationship between dose and blood levels of phosphorus, a potential safety concern for infigratinib treatment.

In addition, two other posters accepted to ENDO 2020, including the design of QED’s PROPEL natural history study in achondroplasia, are available for review on the QED corporate website. Titles for the presentations are:

- **Prospective clinical assessment study in children with achondroplasia: the PROPEL trial**
- **FGFR-selective tyrosine kinase inhibitors, such as infigratinib, show potency and selectivity for FGFR3 at pharmacologically relevant doses for the potential treatment of achondroplasia**

“The currently-enrolling PROPEL natural history study is an important step in establishing a baseline of growth trajectory for children with achondroplasia and will help evaluate whether treatment with infigratinib has a positive effect on growth,” said Melita Irving, Consultant Clinical Geneticist, Guy’s and St Thomas’ Hospital (London). “Fully enrolling the study will help us to advance infigratinib as a potential treatment for children with achondroplasia.”

QED Therapeutics is currently looking to continue enrolling children in PROPEL, a natural history study in children with achondroplasia. The study aims to learn more about the overall health, growth, and possible medical complications in children with achondroplasia. To participate in or learn more about PROPEL, please contact us at: PROPEL@QEDTx.com or visit ClinicalTrials.gov.

Children enrolled in PROPEL may have the opportunity to enroll in PROPEL 2, an interventional phase 2 trial to assess the safety of daily dosing, evidence of efficacy, and dose finding of infigratinib in children with achondroplasia. QED Therapeutics anticipates submitting our IND to the FDA in 2020.

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 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. QED Therapeutics is also evaluating infigratinib for the treatment of achondroplasia. We plan to conduct further clinical trials to evaluate the potential for infigratinib to treat patients with other FGFR-driven tumor types and rare disorders.

For more information on QED Therapeutics, please visit the company’s website at 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

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 potential for a low dose of infigratinib as a treatment option for children with achondroplasia, expectations, plans, and prospects regarding QED Therapeutics' regulatory approval process, clinical trial designs, clinical development plans, clinical trial results, timing and completion of clinical trials, clinical and therapeutic potential of infigratinib, 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, in vitro and in vivo data from prior studies being indicative of future study data, QED Therapeutics' ability to initiate and continue its ongoing and planned clinical trials of infigratinib, QED Therapeutics' ability to enroll children in its ongoing and planned clinical trials, the availability of data from these 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 Annual Report on Form 10-K 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.

QED Contact:

Carolyn Hawley

Canale Communications

carolyn@canalecomm.com

858-354-3581