NEWS RELEASE

bridgebio pharma and affiliate venthera announce dosing of first patient in phase 1/2 clinical trial of bbp-681 for venous, lymphatic, and venolymphatic malformations

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- First-in-human Phase 1/2 trial in patients with venous malformations (VMs), lymphatic malformations (LMs), and venolymphatic malformations (VLMs) associated with PIK3CA or TEK mutations
- Clinical trial enrolling patients across multiple sites in the United States

PALO ALTO, Calif., Feb. 02, 2021 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) and affiliate Venthera, which is focused on developing new treatment options for patients with rare vascular anomalies, today announced that the first patient has been dosed in its Phase 1/2 clinical trial of BBP-681 (also known as VT30 Topical Gel).

This novel investigational therapy is designed to target the genetic drivers of venous (VM), lymphatic (LM), and venolymphatic malformations (VLM) at their source by delivering a potent PI3Kα inhibitor directly to affected tissue. The majority of these cutaneous vascular malformations are driven by somatic mutations in the PIK3CA and TEK genes. These mutations result in overactivation of the PI3K pathway and have been identified as the root cause of the vast majority of VMs, LMs, and VLMs. Because BBP-681 is applied directly to skin lesions, it may minimize complications seen with systemic treatments.

Part 1 of the trial is a four-week treatment, open-label dose escalation study which will determine the dose and regimen of BBP-681 to be carried into Part 2 of the protocol. Part 2 is a 12-week treatment, randomized, placebo-controlled, double-blind, safety and exploratory efficacy study of topically administered BBP-681.

People living with VMs, LMs, and VLMs have significant unmet medical needs related to pain, functional impairment, and disfigurement. Current treatment options include laser ablation, and invasive procedures such as sclerotherapy and surgical excision. BBP-681 is the first potential therapy designed specifically to address the needs of people living with cutaneous forms of these lesions. There are 117,000 people estimated to be living with cutaneous VMs, LMs, and VLMs in the United States and Europe.

“We are dedicated to giving hope to patients by developing new treatment options and working to improve mobility and alleviate discomfort of people living with cutaneous VMs, LMs, and VLMs. The initiation of this study represents an important milestone for the patient community as we translate cutting-edge science into a clinical-stage investigational therapy designed to target these conditions at their known genetic and tissue-specific source," said Thomas M. Rossi, Ph.D., CEO of Venthera.

The Phase 1/2 study is currently enrolling up to 51 patients with VMs, LMs, and VLMs associated with PIK3CA or TEK gene mutations across multiple centers in the U.S. to evaluate the safety and tolerability of BBP-681. The study will also determine the dose and regimen of BBP-681 to be carried into Part 2 of the protocol. Other aims include documenting plasma drug levels of BBP-681 and its active form VT10 and, on an exploratory basis, examining pharmacologic target engagement and change in potential efficacy readouts. For more information, visit **https://clinicaltrials.gov/ct2/show/NCT04409145**.

About Venthera
Venthera is dedicated to improving the lives of patients living with rare vascular anomalies by suppressing the underlying pathways that drive vascular anomaly growth. By targeting the genetic root cause of disease(s), Venthera is seeking to address unmet needs and create a new option for treating VMs, LMs, and VLMs with the hope of reducing the need for invasive and systemic treatments.

As a member of the BridgeBio family, Venthera’s management team is supported by deep expertise in drug discovery, development, and innovation that advances its mission to bring safe and efficient new treatments to patients living with rare vascular anomalies.

About BridgeBio
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 **https://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 and success of Venthera's Phase 1/2 clinical trial of BBP-681/VT30 for the treatment of venous malformations, lymphatic malformations, and venolymphatic malformations associated with PIK3CA or TEK mutations, expectations, plans and prospects regarding Venthera's regulatory approval process for BBP-681/VT30, the ability of BBP-681/VT30 to treat venous malformations, lymphatic malformations, and venolymphatic malformations associated with PIK3CA or TEK mutations in humans, and the timing and success of initial top-line Phase 1/2 data of BBP-681/VT-30, 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, Venthera's ability to continue and complete its Phase 1/2 clinical trial of BBP-681/VT30 for the treatment of venous malformations, lymphatic malformations, and venolymphatic malformations associated with PIK3CA or TEK mutations, past data from preclinical studies not being indicative of future data from clinical trials, Venthera's ability to advance BBP-681/VT30 in clinical development according to its plans, the ability of BBP-681/VT30 to treat venous malformations, lymphatic malformations, and venolymphatic malformations associated with PIK3CA or TEK mutations, as well as those risks set forth in the Risk Factors section of BridgeBio Pharma's most recent Quarterly Report on Form 10-Q and BridgeBio Pharma's other SEC filings. Moreover, Venthera 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.

Contact:
Grace Rauh, BridgeBio Pharma
grace.rauh@bridgebio.com
917-232-5478

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