PhaseBio Announces Successful Pre-BLA Meeting with U.S. FDA for Bentracimab

May 16, 2022

PhaseBio recently received formal written minutes from the FDA following its Pre-BLA Meeting held in early April and is expecting to submit its Biologics License Application (BLA) by early in the fourth quarter of 2022

The FDA indicated willingness to accept a BLA with data from 25-30 uncontrolled bleeding patients, in addition to surgical patients enrolled to date, to potentially support a label that includes both surgical and uncontrolled bleeding indications

To date, PhaseBio has enrolled 35 uncontrolled bleeding patients and has completed enrolling surgical patients in the Phase 3 REVERSE-IT trial

Conference call and webcast today at 8:30 a.m. ET

MALVERN, Pa. & SAN DIEGO--(BUSINESS WIRE)--May 16, 2022-- PhaseBio Pharmaceuticals, Inc. (Nasdaq: PHAS), a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiovascular diseases, today announced the completion of a successful Type B pre-biologics license application (pre-BLA) meeting with the U.S. Food and Drug Administration (FDA) for bentracimab, a recombinant, human monoclonal antibody antigen-binding fragment designed to reverse the antiplatelet activity of ticagrelor in patients presenting with uncontrolled bleeding or in need of surgery. Based on this pre-BLA meeting and the formal written minutes received from the FDA, PhaseBio intends to submit the BLA for bentracimab by early in the fourth quarter of 2022.

During the pre-BLA meeting, the FDA agreed that the company's plans to submit a BLA with data from 25-30 patients with uncontrolled bleeding, together with data from the fully completed surgical cohort, appeared reasonable to support a label with both bleeding and surgical indications but would be a review issue based on the data submitted. To date, and subject to final adjudication, the REVERSE-IT trial has enrolled 35 patients taking ticagrelor who experienced uncontrolled bleeding events. PhaseBio previously intended to base its BLA submission on data from the interim analysis of the Phase 3 REVERSE-IT trial published in December 2021, and the recently presented Phase 2b trial data. Based on the feedback received from the FDA during the pre-BLA meeting, PhaseBio will include in the BLA submission the additional bleeding patients who have enrolled in the REVERSE-IT trial since the pre-specified interim analysis was completed to support a potential bleeding indication.

"This meeting with the FDA marks a significant step forward for the bentracimab development program and PhaseBio," said Jonathan Mow, Chief Executive Officer of PhaseBio Pharmaceuticals. "This is an important moment in the development of bentracimab and a testament to the patients and the commitment and perseverance of the physicians and employees who have worked so diligently on the development of this potentially life-saving medicine. We are rapidly moving ahead with our BLA submission and our pre-commercial and manufacturing efforts to ensure that, if approved, bentracimab will be widely available to patients and caregivers."

John Lee, M.D., Ph.D., Chief Medical Officer of PhaseBio Pharmaceuticals, stated, "We believe the data from our pivotal clinical trials, including the interim analysis of the Phase 3 REVERSE-IT trial, will show that bentracimab can deliver immediate and sustained restoration of platelet function in patients taking ticagrelor who require surgery or experience an uncontrolled bleeding event. We are confident that we have a robust data package for this regulatory submission, and we look forward to continuing to work collaboratively with the FDA to advance the bentracimab BLA submission toward its review as soon as possible."

In addition to discussing the potential allowance of additional bleeding patients into the BLA submission, the FDA also noted that, if during the review process the application was deemed adequate to support approval for only one of the two requested indications, the agency would consider separating and allowing for possible Accelerated Approval of only one of the two indications.

For post-marketing requirements, the FDA confirmed its prior recommendation that the company complete enrollment in the Phase 3 REVERSE-IT trial and submit data from a total of at least 200 patients from this trial, and establish a post-approval registry study that will be active ahead of a product launch following potential Accelerated Approval. PhaseBio plans to continue to enroll patients for the bleeding population to complete the post-approval requirement of completing the REVERSE-IT trial.

Bentracimab has been studied in completed Phase 1 and Phase 2 clinical trials and has demonstrated the potential to bring life-saving therapeutic benefit through immediate and sustained reversal of the antiplatelet activity of ticagrelor, potentially mitigating concerns regarding bleeding risks associated with the use of this antiplatelet drug.

Bentracimab is currently being evaluated in the REVERSE-IT study, a global Phase 3, multi-center, open-label, prospective single-arm trial designed to study reversal of the antiplatelet effects of ticagrelor with bentracimab in patients who present with uncontrolled major or life-threatening bleeding or who require urgent surgery or invasive procedure. Approximately 200 patients are being targeted for enrollment in the REVERSE-IT study at major health centers worldwide. Patients with reported use of ticagrelor within the prior three days who require urgent ticagrelor reversal are eligible for enrollment. A prespecified interim analysis from the REVERSE-IT trial was presented on November 15, 2021, during a late-breaking science session at the American Heart Association's Scientific Sessions 2021. The Company is commencing preparation of a BLA and targeting submission to the FDA early in the fourth quarter of 2022.

Today's Conference Call Information

PhaseBio will host a conference call and webcast today at 8:30 a.m. ET to discuss the Pre-BLA meeting. Analysts and investors can participate in the conference call by dialing (866) 221-1776 for domestic callers and (270) 215-9926 for international callers, using the conference ID 7190686. The webcast can be accessed live on the Events and Presentations page in the Investors section of the PhaseBio website, www.phasebio.com. The webcast will be archived on the company's website for 90 days and will be available for telephonic replay for 14 days following the call by dialing (855) 859-2056 (Domestic) or (404) 537-3406 (International), conference ID 7190686.

About Bentracimab (PB2452)

Bentracimab is a novel, recombinant, human monoclonal antibody antigen-binding fragment designed to reverse the antiplatelet activity of ticagrelor in patients who present with uncontrolled bleeding or require surgery. In a Phase 1 clinical trial, bentracimab demonstrated the potential to bring life-saving therapeutic benefit through immediate and sustained reversal of ticagrelor's antiplatelet activity, mitigating concerns regarding bleeding risks associated with the use of this antiplatelet drug. Data from the Phase 1 clinical trial of bentracimab in healthy volunteers was published in the New England Journal of Medicine in March 2019. In April 2019, bentracimab received Breakthrough Therapy Designation from the FDA. In September 2019, PhaseBio completed a Phase 2a trial in which bentracimab was investigated in healthy, older subjects on dual antiplatelet therapy of ticagrelor and low-dose aspirin. Additionally, the Phase 2a trial investigated a bentracimab regimen for the reversal of supratherapeutic doses of ticagrelor in healthy younger subjects. In November 2021, PhaseBio completed a Phase 2b trial in which bentracimab was investigated in older subjects on dual antiplatelet therapy of ticagrelor and low-dose aspirin, with complete results announced and presented in April 2022. In all active treatment arms in both the Phase 2a and Phase 2b trials, bentracimab achieved immediate and sustained reversal of the antiplatelet effects of ticagrelor and was generally well-tolerated, with only minor adverse events reported. These results are consistent with the results observed in healthy younger subjects treated with ticagrelor in the previously published Phase 1 trial. PhaseBio initiated REVERSE-IT, a pivotal Phase 3 clinical trial of bentracimab, in March 2020 to support a potential Biologics License Application for bentracimab to treat patients with uncontrolled bleeding or requiring surgery. Interim results from the Phase 3 REVERSE-IT trial were presented in November 2021 and subsequently published in NEJM Evidence in December 2021.

About PhaseBio

PhaseBio Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiovascular diseases. The Company's pipeline includes: bentracimab (PB2452), a novel reversal agent for the antiplatelet therapy ticagrelor; and PB6440, an oral agent for the treatment of resistant hypertension. PhaseBio's proprietary elastin-like polypeptide technology platform enables the development of therapies with potential for less-frequent dosing and improved pharmacokinetics, and drives both internal and partnership drug-development opportunities.

PhaseBio is located in Malvern, PA, and San Diego, CA. For more information, please visit www.phasebio.com, and follow us on Twitter @PhaseBio and LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "potential," "projects," "target," "will," "would" and "future" or similar expressions are intended to identify forward-looking statements.

Forward-looking statements include statements concerning or implying the conduct or timing of our clinical trials, including enrollment, and our research, development and regulatory plans for our product candidates, the timing of availability or disclosure of data from those clinical trials and the timing of planned regulatory submissions, the potential for these product candidates to receive regulatory approval from the FDA, EMA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed, marketed and commercialized, including having sufficient product supply at launch, and our ability to complete post-approval requirements. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements.

Risks regarding our business are described in detail in our Securities and Exchange Commission filings, including in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021. These forward-looking statements speak only as of the date hereof, and PhaseBio Pharmaceuticals, Inc. disclaims any obligation to update these statements except as may be required by law.

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