PhaseBio Pharmaceuticals, Inc. Logo

PhaseBio Announces Receipt of Minutes of End-of-Phase 1 Meeting with the FDA and Alignment on a Single, Non-Randomized Phase 3 Trial to Support BLA Submission for PB2452

August 14, 2019

Clinical development plan and Accelerated Approval regulatory path confirmed

Phase 3 trial to initiate in the first quarter of 2020 and intended to support both major bleeding and surgery indications

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

MALVERN, Pa. and SAN DIEGO, Aug. 14, 2019 (GLOBE NEWSWIRE) -- PhaseBio Pharmaceuticals, Inc. (Nasdaq: PHAS), a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for orphan diseases, today announced the receipt of written minutes from the End-of-Phase 1 meeting with the U.S. Food and Drug Administration ("FDA") that was held in July 2019. The End-of-Phase 1 meeting was focused on gaining alignment with the FDA regarding the clinical and regulatory pathway for a potential U.S. approval of PB2452, a novel, recombinant, human monoclonal antibody antigen-binding fragment, or Fab, designed to reverse the antiplatelet activity of ticagrelor in major bleeding and urgent surgery situations. Based on the written minutes from the End-of-Phase 1 meeting, PhaseBio believes that it has reached general agreement with the FDA on the overall design of a single, non-randomized, open label Phase 3 trial of major bleeding and urgent surgical populations to support the submission of a Biologics License Application ("BLA") for potential accelerated approval of PB2452.

End-of-Phase 1 Meeting Details

During the End-of-Phase 1 meeting, the FDA generally agreed with the proposed clinical development plan and agreed that Accelerated Approval was the appropriate approval pathway for PB2452.

To further support safety assessments, the FDA recommended that PhaseBio include 200 active treatment subjects across its Phase 1 and Phase 2 trials, which includes the planned Phase 2b trial that will begin in the fourth quarter of 2019 and run in parallel to the ongoing Phase 2a and planned Phase 3 trial. To date, approximately 50 subjects have received PB2452 in the completed Phase 1 trial and ongoing Phase 2a trial. The Phase 2b trial is expected to enroll a total of 200 subjects, including 150 subjects who will be randomized to receive PB2452.

The FDA also recommended an assessment of PB2452 reversal in patients who may have supratherapeutic blood levels of ticagrelor as a result of ticagrelor overdosage or drug-drug interactions. Based on the pharmacokinetic and pharmacodynamic modeling conducted in earlier preclinical studies and clinical trials of PB2452, PhaseBio believes it has a clear understanding of the appropriate dosing regimen to reverse the antiplatelet effects of supratherapeutic blood levels of ticagrelor and plans to address this request in its ongoing Phase 2a trial, with dosing of these subjects to begin this quarter. PhaseBio expects to complete the Phase 2a trial in the fourth quarter of 2019.

With respect to the pivotal Phase 3 trial design, the FDA agreed with PhaseBio's proposed 200 patient, non-randomized, open-label trial design and the proposed pharmacodynamic, clinical and safety endpoints. The FDA also agreed with the proposed use of the VerifyNow PRUTest® biomarker as the primary endpoint for the Phase 3 trial. PhaseBio has used VerifyNow PRUTest in its Phase 1 and Phase 2a clinical trials, where it demonstrated a high degree of correlation to other biomarkers used to measure platelet function. To support the BLA submission for Accelerated Approval, the FDA recommended that an interim analysis of the Phase 3 trial include data from the first 100 subjects treated with PB2452, with approximately 50 subjects from each of the major bleeding and surgical populations. To support full approval for patients with major bleeding or requiring urgent surgery, the FDA recommended enrollment of 200 total patients in the Phase 3 trial. PhaseBio expects to initiate the Phase 3 trial in the first quarter of 2020; based on an estimated 18-month enrollment timeline, a BLA could potentially be submitted in the second half of 2022. For post-approval commitments, the FDA recommended the completion of the remaining portions of the Phase 3 trial and the establishment of a post-approval registry.

"We are pleased with the constructive and collaborative discussion with FDA officials during the End-of-Phase 1 meeting," said John Lee, M.D., Ph.D., Chief Medical Officer of PhaseBio. "We believe the specific next steps discussed at the meeting, and reflected in the minutes, represent a clear path toward BLA submission. The level of clarity and collaboration we received from the FDA is greatly appreciated. With PB2452 now having a defined development path, coupled with Breakthrough Therapy designation, we believe that we are well positioned to execute our strategy to deliver this potentially life-saving therapy to patients in need as soon as possible."

Today's Conference Call Information

PhaseBio will host a conference call and webcast today at 8:30 a.m. EDT to discuss the End-of-Phase 1 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 5869719. 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 5869719.

About PB2452

PB2452 is a novel, recombinant, human monoclonal antibody Fab fragment designed to reverse the antiplatelet activity of ticagrelor in major bleeding and urgent surgery situations. In a Phase 1 clinical trial, PB2452 demonstrated the potential to bring life-saving therapeutic benefit through immediate and sustained reversal of ticagrelor's anti-platelet activity, mitigating concerns regarding bleeding risks associated with the use of antiplatelet drugs. The Phase 1 clinical trial of PB2452 in healthy volunteers was published in the *New England Journal of Medicine* in March 2019.[1] In April 2019, PB2452 received Breakthrough Therapy designation from FDA. Breakthrough Designation may be granted by FDA when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapy. There are currently no approved reversal agents for ticagrelor or any other antiplatelet drugs.

About PhaseBio

PhaseBio Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies to treat orphan diseases, with an initial focus on cardiopulmonary disorders. The company's lead development candidate is PB2452, a novel reversal agent for the antiplatelet therapy ticagrelor. PhaseBio is also leveraging its proprietary elastin-like polypeptide ("ELP") technology platform to develop therapies with the potential for less-frequent dosing and improved pharmacokinetics. PhaseBio's second product candidate PB1046, which is based on ELP, is a once-weekly vasoactive intestinal peptide receptor agonist being developed for the treatment of pulmonary arterial hypertension.

PhaseBio is located in Malvern, PA and San Diego, CA. For more information, please visit www.phasebio.com.

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," "projects," and "future" or similar expressions are intended to identify forward-looking statements.

Forward-looking statements include statements concerning or implying the conduct, trial design or timing of our clinical trials, timelines for regulatory submissions, and ourresearch, development and regulatory plans for PB2452, PB1046 and our ELP research programs. 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, 2019. 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.

Investor Contact:
John Sharp
PhaseBio Pharmaceuticals, Inc.
Chief Financial Officer
(610) 981-6506
john.sharp@phasebio.com

Media Contact:
Gina Cestari
6 Degrees
(917) 797-7904
gcestari@6degreespr.com

1. Bhatt DL, Pollack CV, Weitz JI, et al. Antibody-Based Ticagrelor Reversal Agent in Healthy Volunteers. *N Engl J Med* 2019;Mar 17.

Source: PhaseBio Pharmaceuticals, Inc.