PhaseBio Launches Clinical Trial to Evaluate PB1046 as a Treatment for Hospitalized COVID-19 Patients

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PhaseBio receives clearance of IND application from U.S. FDA under Coronavirus Treatment Acceleration Program (CTAP)

"VANGARD" trial will assess the efficacy and safety of PB1046 in hospitalized COVID-19 patients at high risk for rapid clinical deterioration and acute respiratory distress syndrome

Based on FDA feedback, PhaseBio believes that positive, clearly interpretable and clinically meaningful trial results may enable PhaseBio to submit a Biologics License Application

MALVERN, Pa. & SAN DIEGO--(BUSINESS WIRE)--May 27, 2020-- PhaseBio Pharmaceuticals, Inc. (Nasdaq: PHAS), a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiopulmonary diseases, today announced FDA authorization to proceed with VANGARD, a potentially pivotal clinical trial to evaluate PB1046 as a treatment for hospitalized COVID-19 patients who are at high risk for rapid clinical deterioration and acute respiratory distress syndrome (ARDS).

PB1046 is a novel, once-weekly, subcutaneously-injected vasoactive intestinal peptide (VIP) receptor agonist that targets VPAC receptors in the cardiovascular, pulmonary and immune systems. VIP is a neurohormone known to have anti-inflammatory, antifibrotic, inotropic, lusitropic and vasodilatory effects and several cardiopulmonary disorders are associated with alterations in levels of VIP or its receptors, VPAC1 and VPAC2. Importantly, VIP has also been observed to have potent bronchodilatory and immunomodulatory effects in the respiratory system. Specifically, VIP has been shown to regulate proinflammatory cytokines including TNF-α, IFN-γ, IL-12, IL-17A and IL-6. In animal models, treatment with VIP peptide prevented acute lung injury and inhibited cytokine-mediated inflammatory responses that are characteristic of ARDS.

"Based on the mechanism of action, the well-documented clinical profile of PB1046, a once-weekly-dosing regimen that eliminates the need for continuous IV infusions required to administer VIP peptide, and evidence of activity of VIP peptide in ARDS, we decided to pursue a rigorous, double-blind randomized trial to evaluate PB1046 as a treatment for COVID-19 patients at high risk of progressing to acute respiratory distress syndrome," said Jonathan Mow, Chief Executive Officer at PhaseBio. "PhaseBio moved very rapidly to develop a study protocol and submit an investigational new drug application (IND) to the FDA. I would like to thank the team at PhaseBio, our network of advisors and investigators and the FDA for the collaborative effort to launch this trial in such an expedited manner."

The VANGARD trial (VIP ANalogue, in hospitalized COVID-19 patients at hiGh risk for rapid clinical deterioration and ARDS) is a multi-center, randomized, double-blind, parallel group clinical trial that will assess the efficacy and safety of once-weekly subcutaneous injections of PB1046 in hospitalized COVID-19 patients at high risk for rapid clinical deterioration and ARDS. Approximately 210 patients will be targeted to be enrolled at approximately 20 sites nationwide. The primary endpoint will measure days alive and free of respiratory failure.

Having received FDA clearance to initiate the VANGARD trial, PhaseBio expects to begin dosing patients by the end of June. Subject to the pace of enrollment and any further impacts from the COVID-19 pandemic, PhaseBio is targeting to report trial results late in the fourth quarter of 2020. Based on feedback from the FDA, PhaseBio believes that positive, clearly interpretable and clinically meaningful results from this trial may enable PhaseBio to submit a Biologics License Application.

"Physicians are in desperate need of new options to treat COVID-19 patients facing rapid deterioration of lung function and before progressing to a ventilator," said John Lee, M.D., Ph.D., Chief Medical Officer at PhaseBio. "The impact of the pandemic on the global healthcare system has been overwhelming during the past few months and the importance of new treatment options that could help reduce this burden cannot be overstated. Based on what we've learned thus far about COVID-19 and acute respiratory distress syndrome, the prognosis for patients who ultimately require ventilator support is exceptionally poor. Early mitigation by PB1046 of the effects of inflammatory cytokines that can cause acute lung injury, is a promising strategy that could prevent patients from declining to the point where they require mechanical ventilation and help alleviate the strain on critical care infrastructure that we're witnessing."

About PB1046

PB1046, a novel, subcutaneously-injected vasoactive intestinal peptide (VIP) analogue, is a recombinant fusion protein composed of VIP and PhaseBio's proprietary elastin-like polypeptide (ELP) biopolymer. Based on the pharmacokinetic profile of PB1046 observed in clinical trials, the fusion of VIP to ELP results in both a prolonged absorption profile and a longer circulating half-life, enabling once-weekly dosing.

PB1046 is in Phase 2 development for the treatment of pulmonary arterial hypertension (PAH) and in a Phase 2 clinical trial for the treatment of hospitalized COVID-19 patients at high risk for rapid clinical deterioration and acute respiratory distress syndrome, which the Company refers to as the VANGARD trial. PhaseBio expects to report initial data from the VANGARD trial in the fourth quarter of 2020, while results from the Phase 2b trial in PAH are expected to be reported in 2021. To date, PB1046 has been administered to more than 70 patients with hypertension or a history of cardiovascular disease in three Phase 1/2 clinical trials conducted in the U.S., with no drug-related serious adverse events reported to date. The U.S. Food and Drug Administration has granted PB1046 orphan drug designation for the treatment of pulmonary arterial hypertension (WHO Group 1 Pulmonary Hypertension) and cardiomyopathy associated with dystrophinopathies.

About PhaseBio

PhaseBio Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiopulmonary diseases. The company's pipeline includes: bentracimab (PB2452), a novel reversal agent for the antiplatelet therapy ticagrelor; PB1046, a once-weekly vasoactive intestinal peptide receptor agonist for the treatment of pulmonary arterial hypertension and acute respiratory distress syndrome; and PB6440, an oral agent for the treatment of resistant hypertension. PhaseBio's proprietary elastin-like polypeptide (ELP) technology platform enables the development of therapies with potential for less-frequent dosing and improved pharmacokinetics, including PB1046, 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.

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 or timing of our VANGARD trial of PB1046 for the treatment of COVID-19 patients at high risk for rapid clinical deterioration and ARDS, or other product candidates in our pipeline, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, including whether the FDA will accept the results of VANGARD for submission of a Biologics License Application, and whether, if approved, these product candidates will be successfully distributed and marketed. 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, 2020. Such risks may be amplified by the impacts of the COVID-19 pandemic. 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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