Phathom Pharmaceuticals Announces Positive Topline Results from Phase 3 PHALCON-NERD-301 Trial Evaluating Daily Dosing of Vonoprazan for Symptomatic Non-Erosive Gastroesophageal Reflux Disease (NERD)

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- Both vonoprazan 10 mg and 20 mg doses met the primary endpoint and showed highly statistically significant greater percentage of 24-hour heartburn free days as compared to placebo ($p<0.0001$)
- Data expected to provide the basis for future regulatory submission for vonoprazan as a daily treatment for patients with NERD, pending completion of 20-week safety extension period
- Over 45 million people in the U.S. are estimated to have NERD, the largest subcategory of gastroesophageal reflux disease (GERD)

FLORHAM PARK, N.J., Jan. 08, 2023 (GLOBE NEWSWIRE) -- Phathom Pharmaceuticals, Inc. (Nasdaq: PHAT), a biopharmaceutical company focused on developing and commercializing novel treatments for gastrointestinal diseases and disorders, announced today positive topline results from PHALCON-NERD-301, a Phase 3 study evaluating the efficacy and safety of vonoprazan for the daily treatment of adults in symptomatic non-erosive gastroesophageal reflux disease (sGERD or NERD). Vonoprazan is an investigational first-in-class potassium-competitive acid blocker (PCAB) from a novel class of medicines that block acid secretion in the stomach. Full results from the study are expected later this year.

The topline results from the four-week, double-blind, placebo-controlled period showed both doses of vonoprazan 10 mg and 20 mg met the primary endpoint and demonstrated significantly greater percentage of 24-hour heartburn-free days versus placebo (mean 46.4% vonoprazan 10 mg, 46.0% vonoprazan 20 mg, compared to 27.5% for placebo; $p<0.0001$ for both vonoprazan 10 mg and 20 mg versus placebo). The median percentage of 24-hour heartburn-free days was 48.3%, 46.7% and 17.0% for vonoprazan 10 mg, vonoprazan 20 mg, and placebo, respectively.

“These data further demonstrate vonoprazan’s potential as a daily therapy in patients with NERD,” said Philip O. Katz, MD, Professor of Medicine and Director of Motility Laboratories at Weill Cornell Medicine, New York City. “NERD is a common cause of reflux-related symptoms and some patients with NERD fail to respond adequately to current therapies. As a practicing physician, I am excited about these data and the role this novel therapy may play in helping to address the needs of patients with symptomatic non-erosive gastroesophageal reflux disease.”

NERD is the largest subcategory of gastroesophageal reflux disease (GERD) and is characterized by reflux-related symptoms in the absence of esophageal mucosal erosions. There are estimated to be over 65 million individuals with GERD in the U.S., and it is estimated that 70% of this population have NERD.

The primary endpoint of the double-blind Phase 3 PHALCON-NERD-301 study evaluated the efficacy of vonoprazan 10 mg and 20 mg as a daily dosing (QD) treatment, as compared to placebo (QD), in the relief of heartburn over four weeks in participants with symptomatic NERD. The trial also includes a blinded 20-week long-term extension period, which is currently ongoing, to further evaluate the safety and efficacy of both doses of vonoprazan after six months of continuous use. A total of 776 patients with symptomatic NERD were enrolled and randomized in the multisite U.S. trial. Full results from the trial are expected to be presented later in 2023.

Vonoprazan was generally well tolerated in the initial four-week double-blind, placebo-controlled phase of the trial. The overall adverse events for all vonoprazan arms were comparable to placebo and consistent with what was reported in previous studies. The most commonly reported adverse event was nausea (2.3% vonoprazan 10 mg, 3.1% vonoprazan 20 mg, 0.4% placebo) with no other events reported above 3.0% in either vonoprazan dose arm. Full safety data from the study will be available following the completion of the 20-week long-term extension period.

“Today’s results mark Phathom’s third positive Phase 3 trial for vonoprazan across three different indications. The topline data are expected to form the basis of our FDA submission seeking approval of vonoprazan as a daily treatment for patients with NERD, following completion of the long-term extension portion of the trial,” said Terrie Curran, Phathom’s President and Chief Executive Officer. “These data are very encouraging for the treatment of NERD, a highly prevalent category of gastroesophageal reflux disease that causes disruptive day and nighttime symptoms for over 45 million patients in the U.S.”

Phathom is currently in discussions with the FDA on a separate Phase 3 trial design to evaluate the novel dosing regimen for vonoprazan as an on-demand or “as needed” treatment for episodic heartburn relief in patients with symptomatic NERD, a dosing treatment regimen not approved in the U.S. for proton pump inhibitors (PPIs). Positive results from Phathom’s Phase 2 PHALCON-NERD on-demand trial were previously presented at the American College of Gastroenterology (ACG) 2022 Annual Scientific Meeting.

About Symptomatic Non-Erosive Gastroesophageal Reflux Disease

sGERD or NERD is the largest subcategory of gastroesophageal reflux disease (GERD) and is characterized by reflux-related symptoms in the absence of esophageal mucosal erosions. There are over 65 million U.S. patients living with GERD, and it is estimated that approximately seventy percent (70%) of this population have NERD. Symptoms of NERD impact overall quality of life and can include episodic heartburn, especially at night, regurgitation, problems swallowing, and chest pain.

About Phathom Pharmaceuticals, Inc.

Phathom Pharmaceuticals is a biopharmaceutical company focused on the development and commercialization of novel treatments for gastrointestinal diseases and disorders. Phathom in-licensed the exclusive rights in the United States, Europe, and Canada to vonoprazan, a first-in-class potassium-competitive acid blocker (PCAB). Vonoprazan-based regimens are approved in the U.S. as part of a co-packaged product in combination with antibiotics for the treatment of *H. pylori* infection in adults, marketed as VOQUEZNA™ TRIPLE PAK™ (vonoprazan, amoxicillin, clarithromycin) and VOQUEZNA™ DUAL PAK™ (vonoprazan, amoxicillin). Phathom is also studying the use of vonoprazan for the treatment of non-erosive reflux disease (NERD). For more information about Phathom, visit the Company's website at www.phathompharma.com and follow the Company on LinkedIn and Twitter.

Forward Looking Statements

Phathom cautions you that statements contained in this press release regarding matters that are not historical facts are forward-looking statements. These statements are based on the Company's current beliefs and expectations. Such forward-looking statements include, but are not limited to, statements regarding the expected timing of full trial results from NERD-301, NERD-301 data providing the basis for submission of a marketing application to FDA and the potential of vonoprazan as a treatment for patients with symptomatic NERD. The inclusion of forward-looking statements should not be regarded as a representation by Phathom that any of its plans will be achieved. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in Phathom's business, including, without limitation: reported topline data is based on preliminary analysis of key efficacy and safety data, which is subject to completion of the NERD-301 trial and additional audit and verification procedures that could result in material changes in the final data; FDA actions related to Phathom's EE NDA or its approved *H. Pylori* NDA may delay or negatively affect the symptomatic NERD program; the FDA may disagree that the existing safety and efficacy data are sufficient for an NDA submission for symptomatic NERD; the inherent risks of clinical development of vonoprazan; Phathom's dependence on third parties in connection with product manufacturing, research and preclinical and clinical testing; regulatory developments in the United States and foreign countries; unexpected adverse side effects or inadequate efficacy of vonoprazan that may limit its development, regulatory approval and/or commercialization, or may result in recalls or product liability claims; Phathom's ability to access additional capital under its term loan facility and royalty interest finance agreements is subject to certain conditions; Phathom's ability to obtain and maintain intellectual property protection for vonoprazan; Phathom's ability to comply with its license agreement with Takeda; Phathom's ability to maintain undisrupted business operations due to the ongoing spread of the COVID-19 coronavirus, including delaying or otherwise disrupting its clinical trials, manufacturing and supply chain, and other risks described in the Company's prior press releases and the Company's filings with the Securities and Exchange Commission (SEC), including under the heading "Risk Factors" in the Company's Annual Report on Form 10-K and any subsequent filings with the SEC. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and Phathom undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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