Phathom Pharmaceuticals Announces Vonoprazan NDA Submission for Non-Erosive GERD

September 26, 2023

- Submission based on positive results from Phase 3 PHALCON-NERD-301 in which vonoprazan 10 mg and 20 mg controlled heartburn symptoms through the entire 6 months of the study with a safety profile consistent with prior vonoprazan studies
- New drug application (NDA) seeks U.S. Food and Drug Administration (FDA) approval for vonoprazan as a daily treatment for Non-Erosive GERD, the largest subcategory of GERD with an estimated U.S. adult population of 38 million
- FDA action date for Non-Erosive GERD NDA expected in the third quarter of 2024

FLORHAM PARK, N.J., Sept. 26, 2023 (GLOBE NEWSWIRE) -- Phathom Pharmaceuticals, Inc. (Nasdaq: PHAT), a biopharmaceutical company focused on developing and commercializing novel treatments for gastrointestinal (GI) diseases, today announced the submission of an NDA to the FDA for vonoprazan as a daily treatment for Non-Erosive gastroesophageal reflux disease (GERD) in adults. The regulatory submission is supported by the positive data from the PHALCON-NERD-301 study, a Phase 3 study evaluating the efficacy and safety of vonoprazan for the daily treatment of adults with Non-Erosive GERD (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.

“The submission of the NERD daily dosing NDA represents a major potential advancement for the estimated 38 million adults in the U.S. suffering from the symptoms of Non-Erosive GERD, many of whom are dissatisfied with currently available therapies,” said Terrie Curran, President and Chief Executive Officer at Phathom. “Our NDA is supported by the results of our successfully completed PHALCON-NERD-301 study, which demonstrated vonoprazan’s durable 24-hour heartburn control over six months and reaffirmed its known safety profile.”

The effectiveness and safety of vonoprazan 10 mg and 20 mg given once daily in NERD was evaluated in a randomized, placebo-controlled, double-blind, 4-week trial with a 20-week blinded extension conducted in the United States in 772 adult patients. As previously reported, both vonoprazan doses met the primary endpoint for the mean percentage of heartburn free days over the 4-week placebo-controlled period at 45%, 44%, and 28% for vonoprazan 10 mg, vonoprazan 20 mg and placebo, respectively ($p<0.0001$ for both doses).

Patients randomized to vonoprazan 10 mg and 20 mg during the initial 4-week period remained on their blinded treatment assignment for the 20-week extension period. The mean percentage of heartburn free days reported over the 20-week extension period for these patients was 63% for vonoprazan 10 mg and 61% for vonoprazan 20 mg.

Additionally, patients who were randomized to placebo during the initial 4-week period were re-randomized to either vonoprazan 10 mg or 20 mg for the 20-week extension period. For these patients, the mean percentage of heartburn free days reported over the 20-week extension period was 62% for vonoprazan 10 mg and 63% for vonoprazan 20 mg.

As previously reported, the overall adverse events were comparable between vonoprazan and placebo during the 4-week placebo-controlled period of which the most common events were nausea, abdominal pain, constipation, and diarrhea, reported at or below 3% for either vonoprazan 10 mg or 20 mg doses. The most common adverse events reported for the two vonoprazan doses during the 20-week extension period were upper respiratory tract infection, sinusitis, influenza, urinary tract infection, nasopharyngitis, nausea, and gastroenteritis, reported at or below 5%.

Phathom expects a 10-month regulatory review and if approved, anticipates a third quarter 2024 U.S. launch for vonoprazan as a daily treatment for Non-Erosive GERD.

Phathom also plans to initiate an additional Phase 3 study to evaluate vonoprazan as an As Needed treatment for episodic heartburn relief in adults with Non-Erosive GERD, a novel dosing treatment regimen.

“While our Non-Erosive daily dosing NDA is under review, we plan to initiate a separate Phase 3 trial studying the As Needed dosing of vonoprazan for active heartburn episodes, a dosing regimen for which proton pump inhibitors (PPIs) are not approved in the U.S., thereby representing a significant unmet need,” said Azmi Nabulsi, M.D., Chief Operating Officer at Phathom. “We look forward to confirming the positive results from our previous Phase 2 As Needed study of vonoprazan in Non-Erosive GERD and, if our Phase 3 study is successful, vonoprazan has the potential to offer a new treatment option for Non-Erosive GERD patients with flexibility to be taken either daily, or for heartburn symptoms that require rapid and sustained relief, on an as-needed basis.”

Vonoprazan is under active review by the FDA as a treatment of Erosive GERD and relief of heartburn associated with Erosive GERD in adults, with a Prescription Drug User Fee Act (PDUFA) goal date of November 17, 2023. If approved, a commercial launch is planned for the fourth quarter of 2023.

About Non-Erosive Gastroesophageal Reflux Disease

Non-Erosive GERD 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 an estimated 38 million U.S. adults living with Non-Erosive GERD, of these approximately 15 million are treated with a prescription medicine annually. 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

Phathom Pharmaceuticals is a biopharmaceutical company focused on the development and commercialization of novel treatments for

gastrointestinal diseases. Phathom has in-licensed the exclusive rights in the United States, Europe, and Canada to vonoprazan, a first-in-class potassium-competitive acid blocker (PCAB). 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

This press release contains forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about the potential of vonoprazan as a treatment for Non-Erosive GERD; the timing of potential approval of the Non-Erosive GERD NDA; the timing of initiating the additional Phase 3 clinical trial to evaluate vonoprazan as an as-needed treatment for episodic heartburn relief in patients with Non-Erosive GERD, and statements regarding the PDUFA goal date and the timing of a U.S. commercial launch for the Erosive GERD and *H. pylori* indications. 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: the FDA may refuse to file the NDA for Non-Erosive GERD; Phathom may delay or choose not to initiate the planned Phase 3 trial to evaluate vonoprazan as an as-needed treatment for Non-Erosive GERD; future data generated from our stability program may be different from the data submitted to the FDA to date and may not demonstrate that our mitigation efforts will continue to maintain the level of the nitrosamine impurity below the acceptable intake (AI) level throughout the shelf life of the products, if approved, which could result in market action or shelf life reduction; risks associated with product manufacturing or formulation changes required to be made in connection with achieving the AI; the FDA may disagree that the existing safety and efficacy data, together with additional data, is sufficient to approve the Erosive GERD NDA or supplements to the *H. pylori* NDAs; 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; 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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