Phathom Pharmaceuticals Announces FDA Approval of VOQUEZNA® (vonoprazan) Tablets for the Relief of Heartburn Associated with Non-Erosive GERD in Adults

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- *VOQUEZNA is now approved and available to treat the largest category of Gastroesophageal Reflux Disease (GERD)*
- *VOQUEZNA met its primary endpoint in its Phase 3 pivotal trial by demonstrating a significant and rapid reduction of heartburn with daily treatment*
- *VOQUEZNA represents the first major innovation in GERD treatment in over 30 years and the only FDA-approved treatment of its kind available in the U.S.*

A Media Snippet accompanying this announcement is available by clicking on this link.

FLORHAM PARK, N.J., July 18, 2024 (GLOBE NEWSWIRE) -- Phathom Pharmaceuticals, Inc. (Nasdaq: PHAT), a biopharmaceutical company focused on developing and commercializing novel treatments for gastrointestinal (GI) diseases, announced today the U.S. Food and Drug Administration (FDA) has approved VOQUEZNA® (vonoprazan) 10 mg tablets for the relief of heartburn associated with Non-Erosive Gastroesophageal Reflux Disease (Non-Erosive GERD) in adults. Non-Erosive GERD represents a substantial segment of the U.S. GERD population, affecting millions of individuals suffering from frequent heartburn. This is the third FDA approval for VOQUEZNA, which is also approved to treat all severities of Erosive Esophagitis (EE), also referred to as Erosive GERD, and in combination with antibiotics for the eradication of *Helicobacter pylori* (*H. pylori*) infection.

“Today marks a significant milestone for millions of GERD patients as we proudly announce the approval of VOQUEZNA for the treatment of Non-Erosive GERD,” said Terrie Curran, President, and Chief Executive Officer at Phathom. “For decades GERD sufferers had no new class of treatment to turn to in the U.S. This approval provides patients and healthcare providers with immediate access to the first and only FDA-approved treatment of its kind, from a new class of acid suppression therapy, and the power to help provide complete 24-hour heartburn-free days and nights. We are very excited to introduce VOQUEZNA to the broader GERD community and look forward to its potential to help change the way this disease is treated.”

Non-Erosive GERD is the largest category of GERD and is characterized by reflux-related symptoms in the absence of esophageal mucosal erosions. An estimated 45 million U.S. adults living with Non-Erosive GERD, and approximately 15 million are treated with a prescription medicine annually. Despite longstanding treatment options, many patients remain dissatisfied with such therapies and continue to suffer from heartburn symptoms which may impact overall quality of life with episodic heartburn, occurring during the day and at night.

“Millions of patients with Non-Erosive GERD continue to suffer from heartburn despite current treatment options,” said Colin W. Howden, M.D., Professor Emeritus, University of Tennessee College of Medicine. “The pivotal study that led to this approval showed that VOQUEZNA significantly reduced heartburn episodes in patients with Non-Erosive GERD along with an established safety profile. Today’s approval of VOQUEZNA provides physicians with a novel, first-in-class treatment that can quickly and significantly reduce heartburn for many adult patients.”

This approval is supported by the positive results from the PHALCON-NERD-301 study (NCT05195528), a Phase 3 randomized, placebo-controlled, double-blind, multi-site U.S. study evaluating the efficacy and safety of VOQUEZNA for the daily treatment of adults with Non-Erosive GERD. The trial enrolled 772 adult patients with Non-Erosive GERD who experienced four or more days of heartburn per week, with the majority having six to seven days of heartburn per week, and compared patients treated with VOQUEZNA 10 mg to placebo in the relief of heartburn over four weeks. The trial also included a 20-week extension period where all patients received VOQUEZNA to evaluate long-term treatment.

In the pivotal trial, VOQUEZNA quickly and significantly reduced heartburn with daily treatment through week 4. VOQUEZNA demonstrated the power of more complete all-day and all-night heartburn-free days with significantly more 24-hour heartburn-free days through week 4 versus placebo, the primary endpoint. The mean percentage of heartburn-free days for patients taking VOQUEZNA was 45% versus 28% for placebo ($p<0.001$), and the median percentage of 24-hour heartburn-free days was 48% versus 17%, respectively. Improvements for those taking VOQUEZNA were also seen in the percentage of each of heartburn-free days and nights, in addition to the percentage of days without rescue antacid use. Results from the pivotal study were previously presented at Digestive Disease Week® (DDW) 2024 and also published in *Clinical Gastroenterology and Hepatology*.

The most common adverse reactions (≥2%) reported in patients treated with VOQUEZNA during the four-week placebo-controlled trial include abdominal pain, constipation, diarrhea, nausea, and urinary tract infection. Upper respiratory tract infection and sinusitis were also reported in patients who received VOQUEZNA in the 20-week extension phase of the trial.

Phathom offers savings programs for eligible patients who face coverage or affordability issues, including co-pay assistance for patients with commercial insurance. For more information, please visit www.voquezna.com/savings.

VOQUEZNA is marketed exclusively by Phathom Pharmaceuticals, Inc. and is currently available via prescription. Please visit www.voquezna.com to learn more about VOQUEZNA.

A Media Snippet accompanying this announcement is available by clicking on this link.

About PHALCON-NERD-301 Study

PHALCON-NERD-301 was a phase 3, randomized, double-blind, multicenter, 4-week study conducted in U.S. patients with heartburn related to Non-Erosive GERD. The primary endpoint was the percentage of days without daytime or nighttime heartburn (24-hour heartburn-free days) over the 4-week placebo-controlled treatment period. The trial also included a 20-week long-term extension period to further evaluate the treatment of VOQUEZNA. A total of 776 patients with Non-Erosive GERD who experienced four or more days of heartburn per week, with the majority having six to seven days of heartburn per week, were enrolled and randomized in the multisite U.S. trial.

About Non-Erosive Gastroesophageal Reflux Disease

Non-Erosive GERD is the largest category of 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 70% of this population have Non-Erosive GERD. Symptoms of Non-Erosive GERD may impact overall quality of life and can include episodic heartburn, especially at night, regurgitation, problems swallowing, and chest pain.

About VOQUEZNA®

VOQUEZNA® (vonoprazan) tablets contain vonoprazan, an oral small molecule potassium-competitive acid blocker (PCAB). PCABs are a novel class of medicines that block acid secretion in the stomach. VOQUEZNA is approved in the U.S. for the treatment of adults with Erosive Esophagitis, also known as Erosive GERD, the relief of heartburn associated with Erosive GERD, the relief of heartburn associated with Non-Erosive GERD, and for the treatment of *H. pylori* infection in combination with either amoxicillin or amoxicillin and clarithromycin. Phathom in-licensed the U.S. rights to vonoprazan from Takeda, which markets the product in Japan and numerous other countries in Asia and Latin America.

About Phathom Pharmaceuticals, Inc.

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 to vonoprazan, a first-in-class potassium-competitive acid blocker (PCAB) that is currently marketed in the United States as VOQUEZNA® (vonoprazan) tablets for the treatment of heartburn associated with Non-Erosive GERD in adults, the healing and maintenance of healing of Erosive GERD in adults and associated heartburn, in addition to VOQUEZNA® TRIPLE PAK® (vonoprazan tablets, amoxicillin capsules, clarithromycin tablets) and VOQUEZNA® DUAL PAK® (vonoprazan tablets, amoxicillin capsules) for the treatment of *H. pylori* infection in adults. For more information about Phathom, visit the company's website at www.phathompharma.com and follow on LinkedIn and X.

INDICATION AND IMPORTANT SAFETY INFORMATION

What is VOQUEZNA?

- VOQUEZNA® (vonoprazan) is a prescription medicine used in adults:
  - for 8 weeks to heal acid-related damage to the lining of the esophagus (called Erosive Esophagitis) and for relief of heartburn related to Erosive Esophagitis.
  - for up to 6 months to maintain healing of Erosive Esophagitis and for relief of heartburn related to Erosive Esophagitis.
  - for 4 weeks for relief of heartburn related to gastroesophageal reflux disease (GERD).
  - for 14 days with the antibiotics amoxicillin and clarithromycin to treat an infection caused by bacteria called *Helicobacter pylori (H. pylori)*.
  - for 14 days with the antibiotic amoxicillin to treat an infection caused by bacteria called H. pylori.

It is not known if VOQUEZNA is safe and effective in children.

Do not take VOQUEZNA if you:

- are allergic to vonoprazan or any of the other ingredients in VOQUEZNA. Allergic reaction symptoms may include trouble breathing, rash, itching, and swelling of your face, lips, tongue or throat.
- are taking a medicine that contains rilpivirine (EDURANT, COMPLERA, JULUCA, ODEFSEY) used to treat HIV-1 (Human Immunodeficiency Virus).

Before taking VOQUEZNA, tell your healthcare provider about all your medical conditions, including if you:

- have low magnesium, calcium, or potassium in your blood, or you are taking a medicine to increase urine (diuretic).
- have kidney or liver problems.
- are pregnant, think you may be pregnant, or plan to become pregnant. It is not known if VOQUEZNA will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if VOQUEZNA passes into your breast milk. You and your healthcare provider should decide if you will take VOQUEZNA or breastfeed. You should not do both.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

VOQUEZNA may affect how other medicines work, and other medicines may affect how VOQUEZNA works. Especially tell your healthcare provider if you take medicine that contains rilpivirine (EDURANT, COMPLERA, JULUCA, ODEFSEY).

What are the possible side effects of VOQUEZNA?

VOQUEZNA may cause serious side effects including:

- **A type of kidney problem (acute tubulointerstitial nephritis):** Some people who take VOQUEZNA may develop a kidney problem called acute tubulointerstitial nephritis. Call your healthcare provider right away if you have a decrease in the amount that you urinate or if you notice blood in your urine.
- **Diarrhea caused by an infection (Clostridioides difficile) in your intestines:** Call your healthcare provider right away if you have watery stools, stomach pain, and fever that does not go away.
- **Bone fractures (hip, wrist, or spine):** Bone fractures in the hip, wrist, or spine may happen in people who take multiple daily doses of another type of medicine that reduces acid in your stomach known as proton pump inhibitors (PPI medicines) for a long period of time (a year or longer). Tell your healthcare provider if you have a bone fracture, especially in the hip, wrist, or spine.
- **Severe skin reactions:** VOQUEZNA can cause rare, but severe skin reactions that may affect any part of your body. These serious skin reactions may need to be treated in a hospital and may be life threatening:
    - Skin rash which may have blistering, peeling, or bleeding on any part of your skin.
    - You may also have fever, chills, body aches, shortness of breath, or enlarged lymph nodes.

If you experience any of these symptoms, stop taking VOQUEZNA and call your healthcare provider right away. These symptoms may be the first sign of a severe skin reaction.

- **Low vitamin B-12 levels:** VOQUEZNA lowers the amount of acid in your stomach. Stomach acid is needed to absorb Vitamin B12 properly. Tell your healthcare provider if you have symptoms of low vitamin B12 levels, including irregular heartbeat, shortness of breath, lightheadedness, tingling or numbness in the arms or legs, muscle weakness, pale skin, feeling tired, or mood changes. Talk with your healthcare provider about the risk of low vitamin B12 levels if you have been on VOQUEZNA for a long time.
- **Low magnesium levels in the body** can happen in people who take VOQUEZNA. Tell your healthcare provider right away if you have symptoms of low magnesium levels, including seizures, dizziness, irregular heartbeat, jitteriness, muscle aches or weakness, or spasms of the hands, feet, or voice.
- **Stomach growths (fundic gland polyps):** A certain type of stomach growth called fundic gland polyps may happen in people who take another type of medicine that reduces acid in your stomach known as proton pump inhibitors (PPI medicines) for a long time. Talk with your healthcare provider about the possibility of fundic gland polyps if you have been on VOQUEZNA for a long time.

The most common side effects of VOQUEZNA for treatment of Erosive Esophagitis and/or relief of heartburn related to gastroesophageal reflux disease include:

- stomach inflammation
- diarrhea
- stomach bloating
- stomach pain
- nausea
- indigestion
- constipation
- high blood pressure
- urinary tract infection

The most common side effects of VOQUEZNA when used with antibiotics for treatment of H. pylori infection include:

- diarrhea
- temporary changes in sense of taste
- vaginal yeast infection
- stomach pain
- headache
- high blood pressure
- cold-like symptoms

These are not all the possible side effects of VOQUEZNA. For more information, ask your healthcare provider or pharmacist. Call your healthcare provider for medical advice about side effects.

You are encouraged to report suspected adverse reactions by contacting Phathom Pharmaceuticals at 1-888-775-PHAT (7428) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see Patient Information and full Prescribing Information for VOQUEZNA.

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 patient access and continued expansion in commercial coverage with other payers; and the Company's estimates of the number of patients with Non-Erosive GERD. 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: Phathom's estimates regarding patient population and commercial coverage could prove to be inaccurate; we may not be able to successfully commercialize VOQUEZNA, which will depend on a number of factors including coverage and reimbursement levels from governmental authorities and health insurers as well as market acceptance by healthcare providers; 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 and non-patent regulatory exclusivity 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 most recent 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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