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

November 1, 2023

- FDA approval marks the first major innovation to the U.S. Erosive GERD market in over 30 years
- VOQUEZNA® met the primary endpoints and key secondary superiority endpoints in the pivotal Phase 3 PHALCON-EE trial evaluating VOQUEZNA in comparison to a standard-of-care proton pump inhibitor (PPI)[1]
- Commercial availability of VOQUEZNA expected December 2023
- Erosive GERD approval provides Phathom $175 million under its revenue interest financing agreement
- Conference call and webcast scheduled for November 2, 2023, at 11:00 a.m. ET

FLORHAM PARK, N.J., Nov. 01, 2023 (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) tablets 10 mg and 20 mg, a novel potassium-competitive acid blocker (PCAB), as a new treatment for adults for the healing of all grades of Erosive Esophagitis, also known as Erosive GERD (gastroesophageal reflux disease), maintenance of healing of all grades of Erosive GERD, and relief of heartburn associated with Erosive GERD.[2]

“This approval demonstrates Phathom's commitment to changing the GI treatment landscape for patients and healthcare providers, bringing the first major innovation to the U.S. Erosive GERD market in over 30 years,” said Terrie Curran, President and Chief Executive Officer at Phathom. “Erosive GERD can be extremely painful and often has a significant impact on patients. Research has shown patients and healthcare providers are largely unsatisfied with current treatments and we are excited about the approval of a first-in-class treatment option that has the potential to meet a large unmet medical need.”

Erosive GERD, also referred to as Erosive Esophagitis or Erosive Acid Reflux, is a major type of GERD that affects approximately 20 million people in the U.S.[3,4] In addition to experiencing troubling heartburn symptoms, patients with inadequately treated Erosive GERD may develop more severe diseases including Barrett’s esophagus, a condition in which esophageal tissue changes can progress to cancer. [3]

This approval is based on positive results from the Phase 3 PHALCON-EE study (NCT04124926). The pivotal trial was a randomized, double-blind, multicenter study that enrolled 1,024 patients with Erosive GERD in the U.S. and Europe and compared VOQUEZNA to the PPI lansoprazole in the healing and maintenance of healing of Erosive GERD and associated heartburn symptom relief.[1]

Results showed that VOQUEZNA 20 mg met the primary endpoint of non-inferiority ($p<0.0001$) for complete healing by Week 8 in patients with all grades of Erosive GERD with a healing rate of 93% compared to 85% for lansoprazole 30 mg, with superior rates of healing demonstrated in a secondary endpoint in patients with moderate-to-severe disease (LA Grade C/D) at Week 2 compared to lansoprazole (70% for VOQUEZNA 20 mg and 53% for lansoprazole 30 mg) ($p=0.0008$). VOQUEZNA 20 mg also demonstrated non-inferiority to lansoprazole 30 mg in the mean percentage of 24-hour heartburn free days over the healing period. In the maintenance phase of the trial, VOQUEZNA 10 mg was superior to lansoprazole 15 mg in maintaining healing at six months in all randomized patients (79% for VOQUEZNA 10 mg, compared to 72% for lansoprazole 15 mg) as well as in the subset of patients with moderate-to-severe Erosive GERD (75% for VOQUEZNA 10 mg, compared to 61% for lansoprazole 15 mg) ($p=0.0490$). In addition, VOQUEZNA 10 mg was evaluated as a secondary endpoint for relief of heartburn in Erosive GERD patients and demonstrated non-inferiority to lansoprazole 15 mg over six months.

Adverse event (AE) rates for VOQUEZNA were comparable to lansoprazole in the trial. The most common AEs in the healing phase (≥ 2% in the VOQUEZNA treatment arm) were gastritis (3.0% for VOQUEZNA 20 mg and 2.0% for lansoprazole 30 mg), diarrhea (2.0% for VOQUEZNA 20 mg and 3.0% for lansoprazole 30 mg), abdominal distension (2.0% for VOQUEZNA 20 mg and 1.0 % for lansoprazole 30 mg), abdominal pain (2.0% for VOQUEZNA 20 mg and 1.0% for lansoprazole 30 mg) and nausea (2.0% for VOQUEZNA 20 mg and 1.0% for lansoprazole 30 mg). The most common AEs in the maintenance phase (≥ 3% in the VOQUEZNA treatment arm) for VOQUEZNA 10 mg compared to lansoprazole 15 mg were gastritis (6.0% vs. 3.0%), abdominal pain (4.0% vs. 2.0%), dyspepsia (4.0% vs. 3.0%), hypertension (3.0% vs. 2.0%), and urinary tract infection (3.0% vs. 2.0%). These are not all of the potential side effects associated with the use of VOQUEZNA. Please see Important Safety information below and the full Prescribing Information for VOQUEZNA for more information.

“For many GERD patients with Erosive Esophagitis, the response to current treatment is suboptimal, leaving them with incomplete healing and ongoing symptoms[5],” said Colin W. Howden, MD, Professor Emeritus, University of Tennessee College of Medicine. “The FDA approval of VOQUEZNA (vonoprazan) provides healthcare providers with a new first-in-class therapeutic option that demonstrated faster healing in the more difficult to treat GERD patients with Erosive Esophagitis. In addition, VOQUEZNA (vonoprazan) provided superior maintenance of healing in all grades of Erosive Esophagitis, compared to lansoprazole, a commonly prescribed PPI, and provided 24-hour heartburn relief on most days in the trial.”

VOQUEZNA is expected to be available in the U.S. in December 2023 and will be marketed exclusively by Phathom Pharmaceuticals, Inc.

Based on the terms of Phathom’s revenue interest financing agreement, the FDA approval of VOQUEZNA for Erosive GERD also entitles the company to receive a $175.0 million payment. This non-dilutive capital will help fund the commercial launch.

Conference Call and Webcast on November 2, 2023, at 11:00 a.m. ET

Phathom will host a live conference call and webcast on Thursday, November 2, 2023, at 11:00 a.m. ET to discuss the FDA approval and the Company's U.S. commercialization plans for Erosive GERD and *H. pylori* infection. The conference call will be available via a listen-only webcast on the investor page of the Company's website at https://investors.phathompharma.com/news-events/events-and-presentations.

About VOQUEZNA®

VOQUEZNA® tablets, VOQUEZNA® TRIPLE PAK® (vonoprazan, amoxicillin, clarithromycin), and VOQUEZNA® DUAL PAK® (vonoprazan, amoxicillin) 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, and VOQUEZNA is the first PCAB to be approved in the U.S. Vonoprazan has shown the potential to provide acid suppression that can achieve pH levels that are important in enhancing treatment effectiveness. Phathom in-licensed the U.S., European, and Canadian rights to vonoprazan from Takeda, which markets the product in Japan and numerous other countries in Asia and Latin America.

About Erosive GERD

Erosive GERD is a major type of gastroesophageal reflux disease (GERD) characterized by erosions in the gastric mucosa caused by acidic reflux of stomach contents into the esophagus.[3] There are estimated to be over 65 million individuals with GERD in the U.S., of which approximately 30% have Erosive GERD.[4,6,7] In addition to experiencing troubling heartburn symptoms, patients with inadequately treated Erosive GERD may progress to more severe diseases including Barrett's esophagus and esophageal cancer. [4]

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 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.

Please see Patient Information and full Prescribing Information for VOQUEZNA.

Visit www.voquezna.com to learn more about VOQUEZNA.

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 or Erosive Acid Reflux) and for relief of heartburn related to Erosive Acid Reflux.
- for up to 6 months to maintain healing of Erosive Acid Reflux and for relief of heartburn related to Erosive Acid Reflux.

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, CABENUVA) 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, CABENUVA).

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 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 Acid Reflux include:

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

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.

Forward Looking Statement

This press release contains forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about the timing of a U.S. commercial launch for vonoprazan for Erosive GERD, the use of proceeds from the revenue interest financing agreement, the size of the Erosive GERD patient population and the potential of vonoprazan to satisfy the large unmet medical need. 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: 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 Company has broad discretion in the use of proceeds from the revenue interest financing agreement; 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 products containing vonoprazan, 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 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.

REFERENCES

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MEDIA CONTACT
Nick Benedetto
1-877-742-8466
media@phathompharma.com

INVESTOR CONTACT
Eric Sciorilli
1-877-742-8466
ir@phathompharma.com

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PDFs accompanying this announcement are available at the links below
VOQUEZNA for Erosive GERD - Fact Sheet
Breaking Down Erosive GERD - Fact Sheet

Photos accompanying this announcement are available at
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