Category

Best Pharmaceutical Product

General Information

Company Name *

Phathom Pharmaceuticals

Product/Solution Name *

Vonoprazan

Compound/Tech Name*

Vonoprazan Fumarate

Trade Name *

VOQUEZNA

Corporate Name *

VOQUEZNA

Date of Approval *

2023-11-01

Indications *

VOQUEZNA is a potassium-competitive acid blocker (PCAB) indicated:

- for relief of heartburn associated with non-erosive gastroesophageal reflux disease in adults.
- for healing of all grades of erosive esophagitis and relief of heartburn associated with erosive esophagitis in adults.
- to maintain healing of all grades of erosive esophagitis and relief of heartburn associated with erosive esophagitis in adults.
- in combination with amoxicillin and clarithromycin for the treatment of Helicobacter pylori (H. pylori) infection in adults.
- in combination with amoxicillin for the treatment of H. pylori infection in adults.

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Therapeutic Areas *

Gastrointestinal (GI), Acid-related Diseases, and Infectious Diseases

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Attached Files:

- PHALCONEE Publication.pdf
- PKPD Benefits of Vonoprazan over PPIs.pdf
- Aliment Pharmacol Ther 2023 Fass Randomised clinical trial Efficacy and safety of ondemand vonoprazan versus.pdf
- Potassiumcompetitive acid blockers rethinking acid suppression for gastroesophageal reflux disease and Helicobacter pylori.pdf
- GERD Infographic Phathom Pharmaceuticals.pdf
- PHAT NERD PDUFA Press Release PRElabel final 71124.docx
- Phathom Pharma VOQUEZNA NonErosive GERD FDA Approval July 2024.docx
- Clincial Gastro and Hep Publication Vonoprazan Efficacy for Heartburn in NonErosive GERD.pdf

Background information and need for drug / device
(please be as specific as possible in your description; limit 500 words)

Background Information and Need for Drug

Gastroesophageal reflux disease (GERD) is one of the most common and burdensome gastrointestinal (GI) conditions in the United States, affecting over 65 million adults. GERD exists in two primary forms: erosive GERD (also known as erosive esophagitis) and non-erosive GERD, both of which can cause chronic heartburn, pain, sleep disruption, and significant reductions in quality of life.

Erosive GERD affects an estimated 20 million Americans and is characterized by inflammation and visible erosions to the lining of the esophagus caused by repeated acid exposure. If inadequately treated, it can lead to complications such as esophageal strictures, Barrett's esophagus, and esophageal cancer. Non-erosive GERD, while lacking visible esophageal damage, can be equally disruptive to patients' daily lives and is often harder to manage with standard therapies due to variability in symptom response.

Despite the high prevalence of both forms of GERD, the treatment landscape has remained largely unchanged for over three decades. Proton pump inhibitors (PPIs), introduced in the late 1980s, remain the standard of care. However, PPIs suffer from well-documented limitations, including delayed onset of action, meal-dependent dosing, and inconsistent acid control, particularly overnight. These pharmacologic limitations contribute to incomplete symptom relief and high rates of treatment dissatisfaction.

Among patients with GERD, 50% switch therapies in search of better symptom control. Only 29% of healthcare providers report satisfaction with current options, and nearly 60% of patients believe their symptoms could be better managed with a novel therapy.[1]

Phathom Pharmaceuticals was founded in 2019 with a clear purpose: to bring meaningful innovation to the acid-related disorder space. In 2023, that mission came to life with the U.S. approval of VOQUEZNA (vonoprazan)-a first-in-class potassium-competitive acid blocker (PCAB)-marking the first new acid suppression treatment approved for GERD in over 30 years.

Unlike PPIs, VOQUEZNA offers rapid onset of action, sustained acid suppression, and predictable, meal-independent dosing. It is now approved for the treatment of both erosive and non-erosive GERD, delivering a new standard for symptom relief and esophageal healing across the spectrum of the disease.

By addressing the clinical limitations of PPIs and meeting the real-world needs of patients with both erosive and non-erosive GERD, VOQUEZNA represents a significant advancement in GI medicine. It offers new hope for patients who have long endured the daily physical and emotional toll of acid-related disorders, and reenergizes a category that had been largely overlooked for more than three decades.

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Attached Files:

- RealWorld Demographic and Clinical Characteristics of VonoprazanTreated US GERD Patients.pdf

History of the development of the solution/product *
(please be as specific as possible in your description; 500 words)

VOQUEZNA (vonoprazan) is an oral, small molecule potassium-competitive acid blocker (PCAB) and the first product from this new class to be approved in the United States for the treatment of gastroesophageal reflux disease (GERD). Developed to overcome the limitations of proton pump inhibitors (PPIs), vonoprazan offers rapid, potent, and meal-independent acid suppression, delivering a meaningful innovation in the treatment of acid-related gastrointestinal disorders.

VOQUEZNA received U.S. FDA approval in November 2023 for the treatment and maintenance of healing of erosive GERD (erosive esophagitis). In July 2024, the FDA approved an additional indication for the treatment of non-erosive GERD, expanding its use across the full spectrum of GERD in adult patients.

The development of vonoprazan was driven by the unmet need for therapies that offer faster and more durable symptom relief than existing PPI-based regimens. Phathom's late co-founder and chairman, Dr. Tachi Yamada, played a foundational role in the molecule's history. As a renowned gastroenterologist and former Chief Medical and Scientific Officer at Takeda Pharmaceuticals, Dr. Yamada helped lead early clinical development of vonoprazan and later co-founded Phathom Pharmaceuticals to bring the therapy to patients in the U.S., Europe, and Canada.

VOQUEZNA's initial U.S. approval was supported by PHALCON-EE, a large, randomized, double-blind, multicenter Phase 3 trial evaluating vonoprazan versus lansoprazole in 1,024 patients with erosive esophagitis. The trial demonstrated:

Superior healing at Week 2 in patients with moderate-to-severe disease (LA Grades C and D)

Superior maintenance of healing at six months in all patients, including those with more severe erosions

Sustained and consistent 24-hour heartburn relief for many patients

The second pivotal Phase 3 study, PHALCON-NERD-301, evaluated vonoprazan in patients with non-erosive GERD, a population where PPI therapy often fails. The study met its primary endpoint, showing statistically significant improvements in heartburn relief compared to placebo.

Vonoprazan has also been studied in the treatment of H. pylori infection. In the PHALCON-HP Phase 3 trial, both vonoprazan-based Dual and Triple Pak regimens demonstrated superior H. pylori eradication rates compared to standard-of-care lansoprazole-based triple therapy.

In addition to these late-stage trials, a successful Phase 2 study evaluated vonoprazan for on-demand or as-needed use in GERD, a potential treatment approach that is not feasible with PPIs due to their delayed onset of action. The study demonstrated that vonoprazan may provide rapid symptom relief when taken only at the time of need, further highlighting the versatility and responsiveness of PCAB therapy.

Looking ahead, Phathom is preparing to initiate a new Phase 2 trial evaluating vonoprazan for the treatment of eosinophilic esophagitis (EoE), a chronic immune-mediated condition with significant unmet need and no approved acid-suppressive treatments for long-term use. The planned study will include both adult and adolescent patient populations.

With three successful Phase 3 programs, regulatory approvals across multiple GERD indications and H. pylori infection, and continued research into novel treatment approaches, VOQUEZNA represents a major step forward in the evolution of acid suppression therapy.

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Why this drug or device is innovative, the broad implications for future research, and/or how it will improve the human condition *

VOQUEZNA (vonoprazan) is the first and only potassium-competitive acid blocker (PCAB) approved in the United States, introducing an entirely new class of acid suppression therapy to an area of medicine that had seen no major advancement in more than 30 years. It is a significant step forward from proton pump inhibitors (PPIs), which have long been the standard of care despite well-documented limitations in onset of action, durability, and patient satisfaction.

Now approved for the treatment of both erosive and non-erosive gastroesophageal reflux disease (GERD), VOQUEZNA addresses the full spectrum of a condition that affects over 65 million Americans. It is also approved as part of co-packaged therapies for the treatment of Helicobacter pylori infection, a chronic bacterial infection associated with gastric cancer. Looking ahead, VOQUEZNA has potential use in other acid-related disorders such as eosinophilic esophagitis (EoE) and functional dyspepsia, expanding its relevance across a wide range of debilitating GI conditions.

The innovation of vonoprazan lies in both its novel mechanism of action and its clinical performance. Unlike PPIs, which require conversion to an active form and must be timed with meals, vonoprazan

directly and reversibly blocks acid secretion at the source. It delivers faster onset, longer-lasting acid control, and more flexible dosing, helping patients achieve consistent symptom relief, including overnight when symptoms are most disruptive.

VOQUEZNA is also the first acid suppressant with clinical data supporting on-demand or as-needed use in GERD. This treatment approach is not feasible with PPIs due to their delayed onset of action. Vonoprazan's fast-acting profile offers new possibilities for personalized treatment, particularly for patients with intermittent or meal-triggered symptoms.

In clinical trials, VOQUEZNA demonstrated superior healing of erosive esophagitis at Week 2, stronger maintenance of healing at six months, and consistent 24-hour heartburn relief. These benefits have translated into strong momentum in the real world. Since launch, the product has seen growing adoption across both specialists and primary care, supported by enthusiastic feedback. Physicians frequently describe VOQUEZNA as a "game changer", and patients report rapid improvements in symptom control and quality of life after switching from PPIs.

The broader implications of VOQUEZNA are significant. By trailblazing a new class of therapy, it resets expectations for acid suppression and reinvigorates clinical research in a space that had been largely overlooked. Its success has already sparked renewed interest in treating other acid-driven diseases that have lacked innovation for years.

VOQUEZNA is not just a new product. It is a foundational shift in how acid-related conditions are understood and treated, offering patients faster, longer-lasting relief and bringing much-needed energy and progress to a long-stagnant field.

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Please provide appropriate references (PubMed, Abstract, Website) *

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