# Phenylketonuria Market Report 2032: Epidemiology, Pipeline Therapies, Latest FDA, EMA, PDMA Approvals by DelveInsight

*According to DelveInsight estimates, in 2022, the highest number of diagnosed Phenylketonuria prevalent cases were observed in the United States among the 7MM.*

LAS VEGAS, NEVADA, UNITED STATES, April 2, 2024 /EINPresswire.com/ -- DelveInsight's "Phenylketonuria Market Insights, Epidemiology, and Market Forecast-2032" report offers an in-depth understanding of the Phenylketonuria, historical and forecasted epidemiology as well as the Phenylketonuria market trends in the United States, EU4 (Germany, Spain, Italy, France) the United Kingdom and Japan.

To Know in detail about the Phenylketonuria market outlook, drug uptake, treatment scenario and epidemiology trends, Click here; Phenylketonuria Market Forecast

Some of the key facts of the Phenylketonuria Market Report:

The Phenylketonuria market size is anticipated to grow with a significant CAGR during the study period (2019-2032).
Phenylketonuria (PKU) is an inborn metabolic defect that can be detected in early life days through routine newborn screening. PKU is defined by the absence or deficiency of an enzyme called phenylalanine hydroxylase (PAH), which is responsible for the amino acid phenylalanine processing.
Newborn blood testing identifies almost all cases of phenylketonuria. All 50 states in the United States require newborns to be screened for PKU. Many other countries also routinely screen infants for PKU. If a person has PKU or family history, the doctor may recommend screening tests before pregnancy or birth. It is possible to identify PKU carriers through a blood test.
According to DelveInsight estimates, in 2022, the highest number of diagnosed prevalent cases of Phenylketonuria (PKU) was observed in the United States among the 7MM.
Most PKU cases are diagnosed in infants due to the high rate of newborn screening.
The pipeline for PKU consists of promising drugs that are anticipated to change the treatment landscape of PKU. The current market anticipates the emergence of PTC923 (PTC Therapeutics) and SYNB1934 (Synlogic) products.
In 2022, the market size of Phenylketonuria (PKU) was found to be ~USD 610 million in the 7MM.
The market size is estimated to increase during the forecast period (2023–2032).
The United States contributed to the largest diagnosed prevalent population of Phenylketonuria

(PKU), acquiring ~36% of the 7MM in 2022.
Among EU4 and the UK, Germany accounted for the highest number of Phenylketonuria (PKU) cases, followed by France, whereas Spain had the lowest number of cases in 2022.
The mutation-specific cases of PKU were further divided into missense mutations, nonsense mutations, mutations at splice sites, deletions, and others. It was found that missense mutations formed the highest number of cases of PKU, whereas the others category formed the least number of cases of PKU, throughout the 7MM, in 2022.
It was found that missense mutations formed the highest number of cases of PKU, i.e., ~11,300 cases, whereas the others category formed the least number of cases of PKU, i.e., ~850 cases in the US, in 2022.
Key Phenylketonuria Companies: PTC Therapeutics, Homology Medicines, Synlogic, BioMarin Pharmaceuticals, and others
Key Phenylketonuria Therapies: KUVAN (Sapropterin Hydrochloride), PALYNZIQ (pegvaliase-pqpz/rAvPAL-PEG/BMN 165), Sepiapterin (PTC923), SYNB1934,  and others
The FDA approved Palynziq (pegvaliase-pqpz) for Phenylketonuria adults in 2018. Palynziq is an injectable enzyme therapy for PKU patients and is manufactured by BioMarin Pharmaceutical.
The Phenylketonuria market share of Kuvan is expected to decrease in the forecast period after losing its market exclusivity and entry of generics in the PKU market.
The Phenylketonuria market will grow as there has been an increase in awareness of the disease with the increasing prevalence. Recently, research and development strategies are being made to produce novel products.
Nevertheless, the Phenylketonuria market will be impeded by the unclear comorbidities associated with the disease and the limitations related to the current treatment.
At present, only two therapies are approved for PKU treatment, and the rest of the management depends on neutral amino acid supplementation and enzyme replacement therapy. With the increase in diagnosis rate and awareness of the disease, the prevalent cases of PKU are expected to increase, which will increase the PKU market size. The pipeline possesses potential drugs as monotherapies and gene therapies, due to which the PKU therapeutics market is expected to grow in the forecast period (2021–2030).
It is expected that PTC-923 will be competing with all the upcoming therapies and already approved therapies in the future. Large pool capture, early market entry, and low safety issues will help PTC-923 capture a significant PKU market share. But compared to HMI-102, the market size of PTC-923 will be less because HMI-102 is gene therapy and will be launched at a much higher price than PTC-923.
The Phenylketonuria market is expected to surge due to the disease's increasing prevalence and awareness during the forecast period. Furthermore, launching various multiple-stage Phenylketonuria pipeline products will significantly revolutionize the Phenylketonuria market dynamics.

Phenylketonuria Overview

Phenylketonuria (PKU) is a genetic disorder affecting metabolism, typically identified through routine newborn screening shortly after birth. It is characterized by a deficiency or absence of

the enzyme phenylalanine hydroxylase (PAH), which is crucial for metabolizing the amino acid phenylalanine. Amino acids serve as fundamental components for building proteins and are vital for normal growth and development. Under normal conditions, PAH converts phenylalanine into another amino acid, tyrosine. However, in individuals with deficient or absent PAH, phenylalanine levels build up, leading to toxicity in the brain. Left untreated, PKU commonly results in severe intellectual disability. The primary treatment approach involves carefully managing the diet to restrict phenylalanine intake, typically starting within the first few days or weeks of life, aiming to prevent intellectual disabilities.

Phenylketonuria Epidemiology

The epidemiology section provides insights into the historical, current, and forecasted epidemiology trends in the seven major countries (7MM) from 2019 to 2032. It helps to recognize the causes of current and forecasted trends by exploring numerous studies and views of key opinion leaders. The epidemiology section also provides a detailed analysis of the diagnosed patient pool and future trends.

Phenylketonuria Epidemiology Segmentation:

The Phenylketonuria market report proffers epidemiological analysis for the study period 2019–2032 in the 7MM segmented into:
Total Prevalence of Phenylketonuria
Prevalent Cases of Phenylketonuria by severity
Gender-specific Prevalence of Phenylketonuria
Diagnosed Cases of Episodic and Chronic Phenylketonuria

Download the report to understand which factors are driving Phenylketonuria epidemiology trends @ Phenylketonuria Epidemiology Forecast

Phenylketonuria Drugs Uptake and Pipeline Development Activities

The drugs uptake section focuses on the rate of uptake of the potential drugs recently launched in the Phenylketonuria market or expected to get launched during the study period. The analysis covers Phenylketonuria market uptake by drugs, patient uptake by therapies, and sales of each drug.
Moreover, the therapeutics assessment section helps understand the drugs with the most rapid uptake and the reasons behind the maximal use of the drugs. Additionally, it compares the drugs based on market share.

The report also covers the Phenylketonuria Pipeline Development Activities. It provides valuable insights about different therapeutic candidates in various stages and the key companies involved in developing targeted therapeutics. It also analyzes recent developments such as collaborations, acquisitions, mergers, licensing patent details, and other information for emerging therapies.

Phenylketonuria Therapies

KUVAN (Sapropterin Hydrochloride)
PALYNZIQ (pegvaliase-pqpz/rAvPAL-PEG/BMN 165)
Sepiapterin (PTC923)
SYNB1934

Phenylketonuria Key Companies

PTC Therapeutics
Homology Medicines
Synlogic
BioMarin Pharmaceuticals

Discover more about therapies set to grab major Phenylketonuria market share @ Phenylketonuria Treatment Landscape

Phenylketonuria Market Outlook

Phenylketonuria (PKU) is a rare genetic disorder characterized by the body's inability to process the amino acid phenylalanine, which serves as a building block for proteins. Although newborns with PKU typically show no symptoms initially, signs of the condition often manifest within a few months if left untreated. Managing PKU involves adhering to a specialized diet and, in some cases, medication to alleviate symptoms and prevent complications.

Early intervention is crucial to mitigate the risk of intellectual impairment. The primary approach to managing PKU involves strict dietary restrictions to limit phenylalanine intake, which may include feeding newborns with PKU breast milk and supplementing with a specific formula like Lofenalac. The appropriate amount of phenylalanine varies for each individual with PKU and may change over time, aiming to provide sufficient intake for optimal growth and bodily functions without exceeding safe levels. However, restricting phenylalanine solely through natural foods is challenging and may compromise overall health, necessitating the use of phenylalanine-free dietary supplements.

Certain protein-rich foods containing phenylalanine, such as meat, milk, fish, and cheese, are typically restricted, while low-protein foods like fruits, vegetables, and select grains are permitted in controlled amounts. Additionally, individuals with PKU should avoid aspartame, a sweetener found in various foods and drinks, as it releases phenylalanine during digestion, leading to elevated blood phenylalanine levels.

Neutral amino acid supplements, available in powder or tablet form, may also be incorporated into the PKU diet to inhibit phenylalanine absorption, particularly for adults with PKU.

Consultation with a healthcare professional or dietitian is recommended to determine the suitability of such supplements for individual dietary needs.

Pharmaceutical interventions for PKU include KUVAN and PALYNZIQ. KUVAN, an oral medication containing BH4, enhances the activity of the residual PAH enzyme, facilitating the conversion of phenylalanine to tyrosine. PALYNZIQ, an injectable enzyme therapy, is reserved for individuals with uncontrolled blood phenylalanine levels despite conventional treatment.

Ongoing research explores additional therapies for PKU, such as large neutral amino acid supplementation and enzyme replacement therapy, aiming to prevent phenylalanine from entering the brain and supplement the deficient enzyme, respectively.

Despite advancements in treatment options, access to effective management remains limited for many PKU patients, leading to intellectual disabilities and other complications. Addressing these challenges requires a comprehensive approach integrating personalized medicine principles and fostering greater awareness and access to innovative treatments.

Scope of the Phenylketonuria Market Report

Study Period: 2019–2032
Coverage: 7MM [The United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom), and Japan]
Key Phenylketonuria Companies: PTC Therapeutics, Homology Medicines, Synlogic, BioMarin Pharmaceuticals, and others
Key Phenylketonuria Therapies: KUVAN (Sapropterin Hydrochloride), PALYNZIQ (pegvaliase-pqpz/rAvPAL-PEG/BMN 165), Sepiapterin (PTC923), SYNB1934, and others
Phenylketonuria Therapeutic Assessment: Phenylketonuria current marketed and Phenylketonuria emerging therapies
Phenylketonuria Market Dynamics: Phenylketonuria market drivers and Phenylketonuria market barriers
Competitive Intelligence Analysis: SWOT analysis, PESTLE analysis, Porter’s five forces, BCG Matrix, Market entry strategies
Phenylketonuria Unmet Needs, KOL’s views, Analyst’s views, Phenylketonuria Market Access and Reimbursement

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Phenylketonuria Pipeline

"Phenylketonuria Pipeline Insight, 2024" report by DelveInsight outlines comprehensive insights of present clinical development scenarios and growth prospects across the Phenylketonuria market. A detailed picture of the Phenylketonuria pipeline landscape is provided, which includes the disease overview and Phenylketonuria treatment guidelines.

Phenylketonuria Epidemiology

DelveInsight's 'Phenylketonuria Epidemiology Forecast to 2032' report delivers an in-depth understanding of the disease, historical and forecasted Phenylketonuria epidemiology in the 7MM, i.e., the United States, EU5 (Germany, Spain, Italy, France, and the United Kingdom), and Japan.

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DelveInsight is a leading Healthcare Business Consultant, and Market Research firm focused exclusively on life sciences. It supports Pharma companies by providing comprehensive end-to-end solutions to improve their performance.

It also offers Healthcare Consulting Services, which benefits in market analysis to accelerate the business growth and overcome challenges with a practical approach.

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