

# Phenylketonuria Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034

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## Abstracts

The 7 major phenylketonuria markets reached a value of US\$ 956.5 Million in 2023. Looking forward, IMARC Group expects the 7MM to reach US\$ 4,734.0 Million by 2034, exhibiting a growth rate (CAGR) of 15.65% during 2024-2034.

The phenylketonuria market has been comprehensively analyzed in IMARC's new report titled "Phenylketonuria Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Phenylketonuria is a rare hereditary condition that results in an accumulation of the amino acid phenylalanine in the body. It is caused by an alteration in the phenylalanine hydroxylase (PAH) gene, which aids in the production of the enzyme required to metabolize phenylalanine. In infants, the symptoms of this ailment can vary from mild to severe and may include a musty odor in the breath, urine, or skin, an unusually small head size, hyperactivity, irritability, delayed development, seizures, skin rashes, etc. Additionally, in older children and adults, the disease manifests as intellectual disability, learning difficulties, behavioral problems, such as anxiety and depression, tremors, jerky movements, etc. Phenylketonuria is typically diagnosed through newborn screening, a blood workup, and a laboratory study. In addition to this, genetic testing is utilized to identify gene mutations associated with this condition. The healthcare provider may also perform certain diagnostic tests and procedures, such as a triple-quadrupole mass spectrometer, urinalysis, Guthrie card bacterial inhibition assay, etc., to confirm a diagnosis among patients.

The increasing cases of genetic mutations and the inflating demand for effective medications to treat such conditions are primarily driving the phenylketonuria market. Besides this, the widespread adoption of several drug formulations, including sapropterin dihydrochloride and pegvaliase-pqpz, for reducing blood phenylalanine

levels in patients is acting as another significant growth-inducing factor. Furthermore, the introduction of novel diagnostic techniques, such as tandem mass spectrometry and high-performance liquid chromatography, which can accurately measure blood phenylalanine levels and differentiate between the disease and other conditions that can cause elevated phenylalanine levels, is also bolstering the market growth. Additionally, several key players are exploring the use of gene therapy to restore phenylalanine hydroxylase activity and prevent neurological as well as cognitive complications associated with the ailment. This, in turn, is further creating a positive outlook for the market. Moreover, the ongoing development of numerous biomarkers, including brain-derived neurotrophic factor (BDNF) and oxidative stress markers, for enhancing the accuracy of diagnosis and monitoring of the disorder is expected to drive the phenylketonuria market in the coming years.

IMARC Group's new report provides an exhaustive analysis of the phenylketonuria market in the United States, EU5 (Germany, Spain, Italy, France, and United Kingdom) and Japan. This includes treatment practices, in-market, and pipeline drugs, share of individual therapies, market performance across the seven major markets, market performance of key companies and their drugs, etc. The report also provides the current and future patient pool across the seven major markets. According to the report the United States has the largest patient pool for phenylketonuria and also represents the largest market for its treatment. Furthermore, the current treatment practice/algorithm, market drivers, challenges, opportunities, reimbursement scenario and unmet medical needs, etc. have also been provided in the report. This report is a must-read for manufacturers, investors, business strategists, researchers, consultants, and all those who have any kind of stake or are planning to foray into the phenylketonuria market in any manner.

### Time Period of the Study

Base Year: 2023

Historical Period: 2018-2023

Market Forecast: 2024-2034

### Countries Covered

United States

Germany

France

United Kingdom

Italy  
Spain  
Japan

### Analysis Covered Across Each Country

Historical, current, and future epidemiology scenario

Historical, current, and future performance of the phenylketonuria market

Historical, current, and future performance of various therapeutic categories in the market

Sales of various drugs across the phenylketonuria market

Reimbursement scenario in the market

In-market and pipeline drugs

Competitive Landscape:

This report also provides a detailed analysis of the current phenylketonuria marketed drugs and late-stage pipeline drugs.

### In-Market Drugs

Drug Overview

Mechanism of Action

Regulatory Status

Clinical Trial Results

Drug Uptake and Market Performance

### Late-Stage Pipeline Drugs

Drug Overview

Mechanism of Action

Regulatory Status

Clinical Trial Results

Drug Uptake and Market Performance

\*Kindly note that the drugs in the above table only represent a partial list of marketed/pipeline drugs, and the complete list has been provided in the report.

Key Questions Answered in this Report:

Market Insights

How has the phenylketonuria market performed so far and how will it perform in the coming years?

What are the markets shares of various therapeutic segments in 2023 and how are they expected to perform till 2034?

What was the country-wise size of the phenylketonuria market across the seven major markets in 2023 and what will it look like in 2034?

What is the growth rate of the phenylketonuria market across the seven major markets and what will be the expected growth over the next ten years?

What are the key unmet needs in the market?

### Epidemiology Insights

What is the number of prevalent cases (2018-2034) of phenylketonuria across the seven major markets?

What is the number of prevalent cases (2018-2034) of phenylketonuria by age across the seven major markets?

What is the number of prevalent cases (2018-2034) of phenylketonuria by gender across the seven major markets?

What is the number of prevalent cases (2018-2034) of phenylketonuria by type across the seven major markets?

How many patients are diagnosed (2018-2034) with phenylketonuria across the seven major markets?

What is the size of the phenylketonuria patient pool (2018-2023) across the seven major markets?

What would be the forecasted patient pool (2024-2034) across the seven major markets?

What are the key factors driving the epidemiological trend of phenylketonuria?

What will be the growth rate of patients across the seven major markets?

### Phenylketonuria: Current Treatment Scenario, Marketed Drugs and Emerging Therapies

What are the current marketed drugs and what are their market performance?

What are the key pipeline drugs and how are they expected to perform in the coming years?

How safe are the current marketed drugs and what are their efficacies?

How safe are the late-stage pipeline drugs and what are their efficacies?

What are the current treatment guidelines for phenylketonuria drugs across the seven major markets?

Who are the key companies in the market and what are their market shares?

What are the key mergers and acquisitions, licensing activities, collaborations, etc. related to the phenylketonuria market?

What are the key regulatory events related to the phenylketonuria market?

What is the structure of clinical trial landscape by status related to the phenylketonuria market?

What is the structure of clinical trial landscape by phase related to the phenylketonuria market?

What is the structure of clinical trial landscape by route of administration related to the phenylketonuria market?

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