Phenylketonuria Market Size in the 7MM was 700 USD Million in 2023, estimated DelveInsight

Phenylketonuria Market

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DELHI, DELHI, INDIA, May 29, 2024 /EINPresswire.com/ -- DelveInsight's 'Phenylketonuria Market Insights, Epidemiology, and Market Forecast–2034' report deliver an in-depth understanding of the Phenylketonuria, historical and forecasted epidemiology as well as the PKU market trends in the United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom) and Japan.

Key Takeaways from the Phenylketonuria Market Research Report
• Among the 7MM, the United States had highest number of Phenylketonuria diagnosed prevalent cases with 18,000 cases in 2023.
• The age□specific data revealed that the highest number of patients affected with Phenylketonuria was found in the age group of <14 years.
• The expected launch of potential therapies may increase Phenylketonuria market size in the coming years, assisted by an increase in the diagnosed prevalent population.
• The leading Phenylketonuria companies such as BioMarin Pharmaceutical, Synlogic, PTC Therapeutics, Jnana Therapeutics, Homology Medicines, Inc, Nestlé Health Science, Moderna, SOM Biotech, Agios Pharmaceuticals, APR Applied Pharma Research, American Gene Technologies, Generation Bio, and others.
• Promising Phenylketonuria therapies in the various stages of development include BMN 307, SYNB1934v1, PTC923, JNT-517, HMI-102, HMI-103, and others.

Discover which therapies are expected to grab the Phenylketonuria Market Share @ Phenylketonuria Market Outlook

Phenylketonuria Overview

Phenylketonuria (PKU) is a rare inherited metabolic disorder that affects the way the body processes the amino acid phenylalanine. Normally, an enzyme called phenylalanine hydroxylase converts phenylalanine into another amino acid called tyrosine. The main cause of phenylketonuria is a mutation in the PAH gene, which is responsible for producing phenylalanine hydroxylase. This genetic abnormality is usually passed down from both parents who carry the mutated gene, making phenylketonuria an autosomal recessive condition.

Phenylketonuria Epidemiology Segmentation
- Total Phenylketonuria Diagnosed Prevalent Cases
- Phenylketonuria Mutation Type-specific Cases
- Phenylketonuria Age-Specific Cases
- Phenylketonuria Severity-Specific Cases

Download the report to understand which factors are driving phenylketonuria epidemiology trends @ Phenylketonuria Epidemiological Insights

Phenylketonuria Treatment Market
Sapropterin dihydrochloride, a synthetic form of tetrahydrobiopterin (BH4), has been introduced as a supplemental treatment to dietary Phe control for Phenylketonuria (PKU). BH4 is a naturally occurring compound cofactor for PAH and other enzymes. Several subsequent studies have found that BH4 supplementation effectively lowers blood serum Phe levels in some individuals with PKU.
KUVAN (sapropterin) is approved for PKU, which is a synthetic form of BH4, the cofactor for phenylalanine hydroxylase (PAH). PAH hydroxylates Phe through an oxidative reaction to form tyrosine. In patients with PKU, PAH activity is absent or deficient. Treatment with BH4 can activate residual PAH enzyme activity, improve the normal oxidative metabolism of Phe, and decrease Phe levels in some patients

Phenylketonuria Therapies and Companies
- BMN 307: BioMarin Pharmaceutical
- SYNB1934v1: Synlogic
- PTC923: PTC Therapeutics
- JNT-517: Jnana Therapeutics
- HMI-102: Homology Medicines, Inc
- HMI-103: Homology Medicines, Inc

Phenylketonuria Market Dynamics
The phenylketonuria market dynamics have been experiencing significant changes over recent years. One key driver of the phenylketonuria market is the increasing awareness and early diagnosis of the disorder. Advances in medical technology and genetic testing have enabled healthcare professionals to identify phenylketonuria cases more accurately and at an earlier stage, allowing for timely interventions and better disease management.

Phenylketonuria Marketed Therapies

- KUVAN (Sapropterin Hydrochloride): Asubio-Pharma/BioMarin-Pharmaceutical

KUVAN is indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin (BH4) responsive PKU and is to be used in conjunction with a Phe-restricted diet. In August 2021, NICE issued final draft guidance which recommends sapropterin (also called KUVAN) as an option for treating PKU in pregnant women until they give birth and for treating the condition in people until they turn 22.

- PALYNZIQ (pegvaliase-pqpz/rAvPAL-PEG/BMN 165): BioMarin Pharmaceutical

PALYNZIQ (pegvaliase-pqpz) injection is the first FDA-approved enzyme substitution therapy for adults with PKU who have uncontrolled blood Phe levels above 600 µmol/L (10 mg/dL) on their current treatment. PALYNZIQ is a once-daily self-administered therapy that acts independently of the phenylalanine hydroxylase (PAH) enzyme, so it is an option for all eligible adult patients living with PKU. In October 2020, the US FDA approved the supplemental biologics license application to increase the maximum allowable dose of 60 mg with PALYNZIQ Injection for treating adults with PKU.

Phenylketonuria Emerging Therapies

- Sepiapterin (PTC923): PTC Therapeutics

PTC923 is an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in the metabolism and synthesis of numerous metabolic products. Sepiapterin reductase plays an enzymatic role in the biosynthesis of tetrahydrobiopterin, which is reported in limited studies to regulate the progression of several tumors.
PTC has submitted an MAA to the EMA for sepiapterin for the treatment of PKU in March 2024. The company also expects to submit an NDA to the FDA for sepiapterin by the third quarter of 2024 and to complete regulatory submissions in Japan in 2024.

- SYNB1934: Synlogic

SYNB1934 is an orally administered, non-systemically absorbed drug candidate being studied as a potential biotherapeutic for phenylketonuria (PKU), an inherited metabolic disease marked by an inability to break down the amino acid phenylalanine (Phe), which can be neurotoxic.

Phenylketonuria Market Outlook

Because newly authorized drugs are often expensive, some patients escape receiving proper treatment or use off-label, less expensive prescriptions. Reimbursement plays a critical role in how innovative treatments can enter the market. The cost of the medicine, compared to the benefit it provides to patients who are being treated, sometimes determines whether or not it will be reimbursed. Regulatory status, target population size, the setting of treatment, unmet needs, the number of incremental benefit claims, and prices can all affect market access and reimbursement possibilities. The report further provides detailed insights on the country-wise accessibility and reimbursement scenarios, cost-effectiveness scenario of approved therapies, programs making accessibility easier and out-of-pocket costs more affordable, insights on

patients insured under federal or state government prescription drug programs, etc.

Phenylketonuria Companies
BioMarin Pharmaceutical, Synlogic, PTC Therapeutics, Jnana Therapeutics, Homology Medicines, Inc, Nestlé Health Science, Moderna, SOM Biotech, Agios Pharmaceuticals, APR Applied Pharma Research, American Gene Technologies, Generation Bio, and others.

Scope of the Phenylketonuria Market Report
- Coverage- 7MM
- Study Period- 2020-2034
- Phenylketonuria Companies- BioMarin Pharmaceutical, Synlogic, PTC Therapeutics, Jnana Therapeutics, Homology Medicines, Inc, Nestlé Health Science, Moderna, SOM Biotech, Agios Pharmaceuticals, APR Applied Pharma Research, American Gene Technologies, Generation Bio, and others.
- Phenylketonuria Therapies- BMN 307, SYNB1934v1, PTC923, JNT-517, HMI-102, HMI-103, and others.
- Phenylketonuria Market Dynamics: Phenylketonuria Market Drivers and Barriers
- Phenylketonuria Unmet Needs, KOL's views, Analyst's views, Phenylketonuria Market Access and Reimbursement

Discover more about Phenylketonuria Drugs in development @ Phenylketonuria Ongoing Clinical Trials Analysis

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