

# Phenylketonuria (PKU) R&D Pipeline Analysis Report, Q4 2020

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## **Abstracts**

Phenylketonuria (PKU) Pipeline Overview

The Q4 Phenylketonuria (PKU) pipeline report is part of the VPA Metabolic Disorder disease pipeline service. This quarterly review describes the pipeline of drugs under development for Phenylketonuria (PKU), provides an update on the mechanism of action and targets, licensing and collaboration opportunities, company profiles including their business information, and market developments.

Phenylketonuria (PKU) Disease Types, Symptoms, Causes, and Available Treatment Options

The comprehensive report on the indication presents Phenylketonuria (PKU) disease overview, Phenylketonuria (PKU) types, Phenylketonuria (PKU) symptoms, causes, and FDA/EMA approved treatment options.

Phenylketonuria (PKU) Pipeline Trends and Insights

The year 2020 witnessed progress in innovations and the number of pipeline agents researched for Phenylketonuria (PKU) indication. The report presents near-term and long-term pipeline development trends and potential insights.

Phenylketonuria (PKU) Company Trends and Insights

The report analyzes the development progress, current status, investments, partnerships, and other developments of 15 companies. Business profiles and contact details of the companies actively perusing Phenylketonuria (PKU) pipeline are assessed.

Phenylketonuria (PKU) R&D Pipeline Development Phase Trends and Insights



From the discovery stage of development to phase 3 development, all the potential pipeline drug candidates are analyzed in the report. Phenylketonuria (PKU) discovery stage, research stage, Phase 1, phase 2, Phase 3, IND enabling studies are provided in the research.

Small Phenylketonuria (PKU) companies look for licensing and collaboration partners High R&D costs and long duration pose challenges for small companies operating in the industry. Further, the presence of large companies and stringent regulations act as barriers for Phenylketonuria (PKU) pipeline development companies. Accordingly, a significant portion of small companies presents potential licensing and collaboration opportunities.

Phenylketonuria (PKU) Pipeline Market News and Developments during 2020 The Phenylketonuria (PKU) industry news, pipeline research, market developments including advancement in phases, pre-clinical study results, clinical trial launches, acquisitions and mergers, regulatory approvals, and others are included in the report.

### Phenylketonuria (PKU) Mechanism of Action and Targets

Leading Mechanism of Action and targets being researched by all the companies and their progress is included. New mechanisms of actions introduced during the year, route of administration, orphan drug designation, and others are provided in the study.

#### New molecular entity details

Drug candidates identified as new molecular entities by FDA, EMA, and other regulators are included in the research.

#### Sources and Research Methodology

Data is updated daily across diseases in the infectious pipeline service. We review our quarterly pipeline reports for the latest 5 years to present longitudinal insights.

#### Scope and Coverage

Phenylketonuria (PKU) pipeline development activities across the early phase, mid-stage, and late phase pipelines including therapeutic candidates

Drug profiles comprising of drug overview, mechanism of action, co-developers, originators, route of administration, molecule type, orphan drug status, new molecular entity, area, and other details.



Company-company partnerships, company-institute partnerships, and investment details of companies are included

15 companies are included including Agios Pharmaceuticals Inc, American Gene Technologies International In, APR Applied Pharma Research SA, BioMarin Pharmaceutical Inc, Codexis Inc, Generation Bio Corp, Homology Medicines Inc, MipSalus ApS, Moderna Therapeutics Inc, Nestle Health Science SA, Pluvia AS, PTC Therapeutics Inc, Sangamo Therapeutics Inc, SOM Biotech SL, Synlogic Inc,

Disease overview, Pipeline trends, market analysis, and other developments

Potential licensing/new business opportunities in Phenylketonuria (PKU) pipeline market

## Highlights

Global coverage of companies and pipeline agents

2020 Trends, market analysis, and developments

Potential growth opportunities

Comprehensive details of drug candidates

#### Reasons to Buy

Drive pipeline research and commercial assessment

Assess most promising drug candidates and stay ahead of the competition

Strengthen your pipeline through identifying business expansion and acquisition opportunities

Develop new candidates based on most focused targets and mechanism of actions



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Codexis Inc

Generation Bio Corp

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Nestle Health Science SA

Pluvia AS

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Sangamo Therapeutics Inc



# SOM Biotech SL Synlogic Inc

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