

# Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Pipeline Review, H2 2018

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# **Abstracts**

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#### SUMMARY

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Phenylalanine 4 hydroxylase or Phenylalanine hydroxylase (PAH) is an enzyme that catalyzes the hydroxylation of the aromatic side-chain of phenylalanine to generate tyrosine. The enzyme works with a molecule called tetrahydrobiopterin (BH4) to carry out this chemical reaction. Tyrosine is used to make several types of hormones, certain chemicals that transmit signals in the brain (neurotransmitters), and a pigment called melanin, which gives hair and skin their color.

Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) pipeline Target constitutes close to 9 molecules. The molecules developed by companies in Phase I and Preclinical stages are 1 and 8 respectively. Report covers products from therapy areas Metabolic Disorders which include indications Phenylketonuria (PKU).

The latest report Phenylalanine 4 Hydroxylase - Pipeline Review, H2 2018, outlays comprehensive information on the Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type. It also reviews key players involved in Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics development with respective active and dormant or discontinued projects.



The report is built using data and information sourced from proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources.

**Note:** Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

#### **SCOPE**

The report provides a snapshot of the global therapeutic landscape for Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1)

The report reviews Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

The report features descriptive drug profiles for the pipeline products which includes, product description, descriptive MoA, R&D brief, licensing and collaboration details & other developmental activities

The report reviews key players involved in Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics and enlists all their major and minor projects

The report assesses Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) targeted therapeutics



#### **REASONS TO BUY**

Gain strategically significant competitor information, analysis, and insights to formulate effective R&D strategies

Identify emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage

Identify and understand the targeted therapy areas and indications for Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1)Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

Plan mergers and acquisitions effectively by identifying key players and it's most promising pipeline therapeutics

Devise corrective measures for pipeline projects by understanding Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) development landscape

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope



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BioMarin Pharmaceutical Inc

Codexis Inc

Homology Medicines Inc

Moderna Therapeutics Inc

Rubius Therapeutics Inc

Synthetic Biologics Inc

Ultragenyx Pharmaceutical Inc

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Featured News & Press Releases

Oct 18, 2018: American Gene Technologies granted FDA orphan drug designation for phenylketonuria

Oct 18, 2018: Homology Medicines Presents Long-Term Efficacy Data of a Single Dose of Gene Therapy Development Candidate for Treatment of Phenylketonuria

Jul 11, 2018: Codexis Appoints Dr. Hicham Alaoui, Veteran Drug Discoverer, as Vice President, Biotherapeutics Research & Development

Jul 09, 2018: Codexis Doses First Subjects in Phase 1a Trial of CDX-6114



May 18, 2018: Homology Medicines Presents Data from PKU Gene Therapy Program Demonstrating Long-Term Durability of Treatment in IND-Enabling Studies Apr 30, 2018: Homology Medicines Announces Upcoming Presentations on its Genetic Medicines Platform at the American Society of Gene & Cell Therapy Meeting Appendix

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#### **COMPANIES MENTIONED**

American Gene Technologies International Inc
BioMarin Pharmaceutical Inc
Codexis Inc
Homology Medicines Inc
Moderna Therapeutics Inc
Rubius Therapeutics Inc
Synthetic Biologics Inc
Ultragenyx Pharmaceutical Inc



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