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Phenylalanine 4 Hydroxylase (Phe 4 Monooxygenase or PAH or EC 1.14.16.1) - Drugs in Development, 2021

https://marketpublishers.com/r/PDA403AA5EC2EN.html
Date: March 2021
Pages: 65
Price: US$ 3,500.00 (Single User License)
ID: PDA403AA5EC2EN

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 10 molecules. The molecules developed by companies in Phase II, Phase I, Preclinical and Discovery stages are 2, 1, 6 and 1 respectively. Report covers products from therapy areas Metabolic Disorders which include indications Phenylketonuria (PKU) and Phenylalanine Hydroxylase Deficiencies.

The latest report Phenylalanine 4 Hydroxylase - Drugs in Development, 2021, 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.

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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

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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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Featured News & Press Releases

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Nov 06, 2020: Homology Medicines announces presentation of positive data from the dose-escalation phase of the pheNIX Gene Therapy trial for adults with PKU
Nov 05, 2020: Homology Medicines announces upcoming oral presentation on pheNIX gene therapy clinical trial for adults with PKU
Oct 02, 2020: BioMarin, pioneer in phenylketonuria (PKU) and gene therapy, receives FDA Fast Track Designation for PKU investigational gene therapy, BMN 307
Sep 24, 2020: BioMarin, pioneer in phenylketonuria (PKU) and gene therapy, doses first participant in global PHEARLESS phase 1/2 study of BMN 307 gene therapy
May 26, 2020: Homology Medicines announces Peer-Reviewed Publication describing molecular characterization of precise In Vivo Nuclease-Free gene editing with PKU program
May 04, 2020: Homology Medicines announces Publication in Genetic Engineering & Biotechnology News on dual gene therapy and nuclease-free gene editing platform for PKU
Apr 29, 2020: Homology Medicines announces presentations at upcoming American Society for Gene & Cell Therapy Annual Meeting
Mar 16, 2020: Homology Medicines announces peer-reviewed publication of HMI-102 investigational gene therapy demonstrating restoration of normal metabolic pathway in PKU disease model
Mar 10, 2020: American Gene Technologies to present at 2020 Inborn Errors of Metabolism Drug Development Summit in Boston
Jan 13, 2020: BioMarin, pioneer in phenylketonuria, to begin clinical trial with BMN 307 gene therapy
Dec 17, 2019: Homology Medicines announces encouraging initial clinical data from its pheNIX Gene Therapy Trial for PKU
Dec 16, 2019: Homology Medicines announces peer-reviewed publication of five-year retrospective study conducted in collaboration with two PKU Key Opinion Leaders demonstrating substantially elevated Phe levels in adults with classical PKU on standard of care
Dec 03, 2019: Homology Medicines announces appointment of Gabriel M. Cohn, M.D., as chief medical officer
Oct 25, 2019: Homology Medicines presents preclinical data from its investigational pku gene editing program, demonstrating phenotypic correction and molecular confirmation of editing precision
Oct 21, 2019: Homology Medicines presents data from investigational MLD gene therapy program HMI-102 at the American Society of Human Genetics (ASHG) 2019 Meeting
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