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Paroxysmal nocturnal haemoglobinuria - Pipeline Insight, 2021

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Abstracts

This report can be delivered to the clients within 72 Hours

DelveInsight's, "Paroxysmal nocturnal haemoglobinuria - Pipeline Insight, 2021," report provides comprehensive insights about 25+ companies and 25+ pipeline drugs in Paroxysmal nocturnal haemoglobinuria pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Paroxysmal nocturnal haemoglobinuria Understanding

Paroxysmal nocturnal haemoglobinuria: Overview

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare acquired, life-threatening disease of the blood. The disease is characterized by complement-mediated hemolysis with or without hemoglobinuria, an increased susceptibility to thrombotic episodes and/or some degree of bone marrow dysfunction. PNH leads to excessive breakdown of red blood cells, leading to the release of a large amount of haemoglobin into the urine. Symptoms and signs of PNH include: fatigue; dark red/brown urine; difficulty swallowing, abdominal pain, infections, and bruising. The principal studies used to establish the diagnosis of PNH are flow cytometry of peripheral blood and bone marrow analysis. PNH typically

starts from the early thirties to the mid?forties, and often persisting for decades, with a continued dependence on blood transfusions in a proportion of patients. The appropriate treatment for PNH depends on the severity of symptoms. The mainstay of PNH treatment is the drug eculizumab (Soliris).

'Paroxysmal nocturnal haemoglobinuria - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Paroxysmal nocturnal haemoglobinuria pipeline landscape is provided which includes the disease overview and Paroxysmal nocturnal haemoglobinuria treatment guidelines. The assessment part of the report embraces, in depth Paroxysmal nocturnal haemoglobinuria commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Paroxysmal nocturnal haemoglobinuria collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Paroxysmal nocturnal haemoglobinuria R&D. The therapies under development are focused on novel approaches to treat/improve Paroxysmal nocturnal haemoglobinuria.

Paroxysmal nocturnal haemoglobinuria Emerging Drugs Chapters

This segment of the Paroxysmal nocturnal haemoglobinuria report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Paroxysmal nocturnal haemoglobinuria Emerging Drugs

Pegcetacoplan (APL-2): Apellis Pharmaceuticals

APL-2 is a synthetic cyclic peptide conjugated to a polyethylene glycol (PEG) polymer

that binds specifically to C3 and C3b, effectively blocking all three pathways of complement activation (classical, lectin, and alternative). In February 2019 Apellis Pharmaceuticals announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to APL-2. In November 2020, Apellis Pharmaceuticals announced that the U.S. Food and Drug Administration (FDA) has accepted and granted Priority Review designation for the New Drug Application (NDA) for pegcetacoplan for the treatment of paroxysmal nocturnal hemoglobinuria (PNH).

Pozelimab: Regeneron Pharmaceuticals

Pozelimab is an investigational, fully-human monoclonal antibody designed to block complement factor C5 and prevent the destruction of red blood cells (hemolysis). It is an IgG4 antibody that binds with high affinity to wild-type and variant human C5 and blocks its activity. Pozelimab was invented using Regeneron's proprietary VelocImmune technology, which uses a unique genetically-humanized mouse to produce optimized fully-human antibodies. The drug is currently under clinical evaluation for the treatment of Paroxysmal nocturnal haemoglobinuria and gastrointestinal disorders.

Further product details are provided in the report

Paroxysmal nocturnal haemoglobinuria: Therapeutic Assessment

This segment of the report provides insights about the different Paroxysmal nocturnal haemoglobinuria drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Paroxysmal nocturnal haemoglobinuria

There are approx. 25+ key companies which are developing the therapies for Paroxysmal nocturnal haemoglobinuria. The companies which have their Paroxysmal nocturnal haemoglobinuria drug candidates in the most advanced stage, i.e. Preregistration include, Apellis Pharmaceuticals.

Phases

DelveInsight’s report covers around 25+ products under different phases of clinical

development like

- Late stage products (Phase III)
- Mid-stage products (Phase II)
- Early-stage product (Phase I) along with the details of
- Pre-clinical and Discovery stage candidates
- Discontinued & Inactive candidates
- Route of Administration

Paroxysmal nocturnal haemoglobinuria pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

- Oral
- Parenteral
- Intravitreal
- Subretinal
- Topical
- Molecule Type

Products have been categorized under various Molecule types such as

- Monoclonal Antibody
- Peptides
- Polymer

- Small molecule
- Gene therapy
- Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Paroxysmal nocturnal haemoglobinuria: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Paroxysmal nocturnal haemoglobinuria therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Paroxysmal nocturnal haemoglobinuria drugs.

Paroxysmal nocturnal haemoglobinuria Report Insights

- Paroxysmal nocturnal haemoglobinuria Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

Paroxysmal nocturnal haemoglobinuria Report Assessment

- Pipeline Product Profiles
- Therapeutic Assessment

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Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Paroxysmal nocturnal haemoglobinuria drugs?

How many Paroxysmal nocturnal haemoglobinuria drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Paroxysmal nocturnal haemoglobinuria?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Paroxysmal nocturnal haemoglobinuria therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Paroxysmal nocturnal haemoglobinuria and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

Alexion Pharmaceuticals

Apellis Pharmaceuticals

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Regeneron Pharmaceuticals

Akari Therapeutics

Chugai Pharmaceutical

Alnylam Pharmaceuticals

BioCryst Pharmaceuticals

Kira Pharmaceuticals

Omeros Corporation

Ra Pharmaceuticals

Turgut Ilacari

Key Products

Vemircopan

Pegcetacoplan (APL-2)

Pozelimab

Nomacopan

Crovalimab

Cemdisiran

BCX-9930

KP-104

MASP-2 therapeutics

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