

Paroxysmal Nocturnal Hemoglobinuria Market Size is expected to grow by 2034, estimates DelveInsight

Paroxysmal Nocturnal Hemoglobinuria Market Insights, Epidemiology, and Market Forecast-2034

DELHI, DELHI, INDIA, May 27, 2024 /EINPresswire.com/ -- DelveInsight's

"Paroxysmal Nocturnal Hemoglobinuria Market Insights, Epidemiology, and Market Forecast – 2034" report delivers an in-depth understanding of the Paroxysmal Nocturnal Hemoglobinuria historical and forecasted epidemiology as well as the market trends in the United States, EU4 (Germany, France, Italy, and Spain) and the United Kingdom, and Japan.



Key Takeaways from the Paroxysmal Nocturnal Hemoglobinuria Market Research Report

- According to Schubert et al. (2022), Paroxysmal Nocturnal Hemoglobinuria is a very rare disease with an estimated prevalence of up to 16 cases/1 million population and an incidence of approximately 1.3 cases/1 million population in the UK.
- According to DelveInsight's estimates, in the US, there were approximately ~2,900 cases of PNH among males, while females accounted for ~3,100 cases in 2023. These cases are anticipated to increase by the end of 2034.
- According to DelveInsight's estimates, In 2023, the total Paroxysmal Nocturnal Hemoglobinuria diagnosed prevalent cases in the US were ~6,100 cases which is anticipated to increase by 2034 at a moderate CAGR during the study period 2024-2034.
- Among the 7MM countries, the US comprised ~49% of the total Paroxysmal Nocturnal Hemoglobinuria diagnosed prevalent cases in 2023.
- The leading Paroxysmal Nocturnal Hemoglobinuria Companies working in the market include Alexion Pharmaceuticals, Apellis Pharmaceuticals, Hoffmann-La Roche, Novartis, Regeneron Pharmaceuticals, RA Pharmaceuticals Inc., and others.
- Promising Paroxysmal Nocturnal Hemoglobinuria Therapies in the various stages of development include Coversin, Eculizumab, Ravulizumab, SB12 (proposed eculizumab

biosimilar), Soliris (eculizumab), and others.

- May 2024:- Alexion Pharmaceuticals Inc.- A Phase 3, Randomized, Open-Label, Active-Controlled Study of ALXN1210 Versus Eculizumab in Complement Inhibitor-Naïve Adult Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH). The primary purpose of this study was to assess the noninferiority of ravulizumab compared to eculizumab in adult participants with PNH who had never been treated with a complement inhibitor (treatment-naïve).
- April 2024:- Novartis Pharmaceuticals- A Multicenter, Single-arm, Open-label Trial to Evaluate Efficacy and Safety of Oral, Twice Daily Iptacopan in Adult PNH Patients Who Are Naïve to Complement Inhibitor Therapy. The purpose of this Phase 3 study was to determine whether iptacopan is efficacious and safe for the treatment of Paroxysmal nocturnal hemoglobinuria (PNH) patients who were naïve to complement inhibitor therapy.

Discover which therapies are expected to grab the Paroxysmal Nocturnal Hemoglobinuria Market Share @ [Paroxysmal Nocturnal Hemoglobinuria Market Outlook](#)

Paroxysmal Nocturnal Hemoglobinuria Overview

Paroxysmal Nocturnal Hemoglobinuria, a rare hematological disorder, exhibits hemolysis, thrombosis, and bone marrow failure, often progressing to myelodysplastic syndromes. Hematologists find its diverse manifestations and intricate pathophysiology fascinating. It manifests as classical anemia from surface molecule defects and aplastic anemia, with some patients experiencing both. Diagnosis usually occurs in a nonacute setting. □Formerly, PNH diagnosis used obsolete Ham and sucrose tests. Now, flow cytometry (FCM) is standard, detecting anomalies with monoclonal antibodies or FLAER, avoiding interference from glycophorin in red blood cells.

Paroxysmal Nocturnal Hemoglobinuria Epidemiology Insights

- Total Paroxysmal Nocturnal Hemoglobinuria Diagnosed Prevalent Cases
- Paroxysmal Nocturnal Hemoglobinuria Gender-specific Cases

Download the report to understand which factors are driving Paroxysmal Nocturnal Hemoglobinuria Epidemiology trends @ [Paroxysmal Nocturnal Hemoglobinuria Epidemiological Insights](#)

Paroxysmal Nocturnal Hemoglobinuria Treatment Landscape

The standard treatment for patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) involves the use of C5 inhibitors, with Eculizumab (SOLIRIS) and Ravulizumab (ULTOMIRIS) being approved monoclonal antibodies that block terminal complement activation at C5, inhibiting the formation of C5a and C5b-9. Both drugs, administered via IV infusion, share the same C5 epitope, but Ravulizumab was engineered for an extended half-life. Pegcetacoplan, the first C3 inhibitor, has also been recently approved by the FDA and EMA. EMPAVELI, an oral Factor B inhibitor, represents another advancement in PNH treatment.

Paroxysmal Nocturnal Hemoglobinuria Emerging Therapy Assessment

Expected Launch of potential therapies may increase the market size in the coming years, assisted by an increase in the diagnosed prevalent population of PNH. Owing to the positive outcomes of the several products during the developmental stage by key players such as Alexion, Bio Cryst pharmaceuticals, Chugai Pharmaceuticals, and others. The PNH market is expected to witness a significant positive shift in the forecast period of 2024-2034.

Paroxysmal Nocturnal Hemoglobinuria Drugs Uptake

- RG6107 or SKY59 (crovalimab): Hoffman la Roche/Chugai Pharmaceuticals

Crovalimab is also known as RG6107, and SKY59 is a humanized complement inhibitor C5 monoclonal antibody (anti-C5 recycling antibody) discovered by Chugai using recycling antibody technology. SKY59 is designed to target C5, a key component of the complement system, and is expected to control complement activity. It inhibits complement activation by blocking the cleavage of c5 to c5a and c5b, which is the cause of PNH. Roche is codeveloping SKY59 with Chugai Pharmaceuticals.

- ALXN2040 (danicopan): Alexion Pharmaceuticals

Danicopan is an investigational, first-in-class, oral proximal factor D inhibitor being developed by Alexion Pharmaceuticals. Factor D (FD), a serine protease, catalyzes the cleavage of complement factor B into Ba and Bb, which allows for the formation of the AP C3 convertase. By inhibiting FD, danicopan, blocks C3 convertase formation, the control point for AP activation, and the amplification of all pathways. This leads to the inhibition of C3 cleavage, C3 fragment deposition, terminal pathway activation, and MAC formation. It is in Phase III.

Paroxysmal Nocturnal Hemoglobinuria Marketed Therapies

- SOLIRIS (eculizumab): AstraZeneca (Alexion Pharmaceuticals)

SOLIRIS was the first therapy approved to treat patients with Paroxysmal Nocturnal Haemoglobinuria (PNH) to reduce hemolysis patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy for treating generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody-positive and in adult patients with neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody-positive.

Eculizumab is a monoclonal antibody that binds to the complement protein C5 with high affinity, thereby inhibiting its cleavage to C5a and C5b and preventing the generation of the terminal complement complex C5b-9. SOLIRIS inhibits terminal complement-mediated intravascular hemolysis in PNH patients and complement-mediated thrombotic microangiopathy (TMA) in patients with aHUS.

- EMPAVELI/ ASPAVELI (pegcetacoplan): Apellis Pharmaceuticals/ Swedish Orphan Biovitrum

Pegcetacoplan is an investigational, targeted C3 inhibitor designed to regulate excessive complement activation, leading to the onset and progression of many serious diseases. Pegcetacoplan is a synthetic cyclic peptide conjugated to a polyethylene glycol polymer that

binds specifically to C3 and C3b. EMPAVELI is a complement inhibitor indicated to treat adult patients with Paroxysmal Nocturnal Haemoglobinuria (PNH).

Pegcetacoplan binds to complement protein C3 and its activation fragment C3b, thereby regulating the cleavage of C3 and the generation of downstream effectors of complement activation. In PNH, extravascular hemolysis (EVH) is facilitated by C3b opsonization, while intravascular hemolysis (IVH) is mediated by the downstream membrane attack complex (MAC). Pegcetacoplan acts proximally in the complement cascade, controlling both C3b-mediated EVH and terminal complement-mediated IVH.

Paroxysmal Nocturnal Hemoglobinuria Market Outlook

Paroxysmal Nocturnal Haemoglobinuria (PNH) is a rare hematological disorder characterized by episodes of hemolysis, thrombosis, and bone marrow failure and sometimes progresses to myelodysplastic syndromes. It has fascinated hematologists for more than a century due to its diverse manifestations and intricate pathophysiology. PNH can sink in 2 ways: one is a classical form called anemia due to hemolysis because of the defect of surface molecules; another is aplastic anemia. In some patients, both of these forms can sink into one another. PNH is typically diagnosed in a nonacute setting.

Scope of the Paroxysmal Nocturnal Hemoglobinuria Market Report

- Coverage- 7MM
- Study Period- 2020-2034
- Forecast Period- 2024-2034
- Paroxysmal Nocturnal Hemoglobinuria Companies- Alexion Pharmaceuticals, Apellis Pharmaceuticals, Hoffmann-La Roche, Novartis, Regeneron Pharmaceuticals, RA Pharmaceuticals Inc., and others.
- Paroxysmal Nocturnal Hemoglobinuria Therapies- Coversin, Eculizumab, Ravulizumab, SB12 (proposed eculizumab biosimilar), Soliris (eculizumab), and others.
- Paroxysmal Nocturnal Hemoglobinuria Market Dynamics: Paroxysmal Nocturnal Hemoglobinuria Market Drivers and Barriers
- Paroxysmal Nocturnal Hemoglobinuria Unmet Needs, KOL's views, Analyst's views, Paroxysmal Nocturnal Hemoglobinuria Market Access and Reimbursement

Discover more about Paroxysmal Nocturnal Hemoglobinuria Drugs in development @

[Paroxysmal Nocturnal Hemoglobinuria Ongoing Clinical Trials Analysis](#)

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