

Global Von-Hippel Lindau Disease Market: Focus on Treatment, Clinical Manifestation, Country and Regional Analysis - Analysis and Forecast, 2025-2035

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Abstracts

Von Hippel-Lindau (VHL) disease is a rare inherited condition that leads to the development of tumors and cysts in various organs, such as the kidneys, central nervous system (CNS), eyes, and pancreas. These tumors may be benign or malignant, with kidney tumors often becoming cancerous. VHL is caused by mutations in the VHL gene, which disrupts the normal regulation of cell growth and blood vessel formation.

The market for VHL treatments is expanding, driven by technological advancements in genetic testing, new treatment options, and increased research focused on understanding the disease. As awareness grows, pharmaceutical and biotechnology companies are developing targeted therapies to address the underlying genetic issues that cause VHL. Despite being rare, the rising number of diagnosed patients globally, combined with the introduction of innovative treatments, is fueling the growth of the VHL market.

The growing availability and sophistication of molecular diagnostic tools, such as next-generation sequencing (NGS), exome sequencing, and whole-genome sequencing, have significantly improved the early detection of Von Hippel-Lindau disease. These tools allow for precise identification of genetic mutations, enabling clinicians to identify individuals at risk for VHL at an earlier stage. Early detection is crucial for the successful management of VHL, particularly in minimizing tumor development and facilitating timely treatment decisions. The development of genetic testing panels and improved bioinformatics for mutation screening further augments the ability to diagnose VHL early, enhancing treatment outcomes.

Targeted therapies, such as Belzutifan (Welireg), have revolutionized the treatment

landscape for VHL patients. Belzutifan specifically targets the HIF-2 α (hypoxia-inducible factor 2-alpha) pathway, which plays a pivotal role in the tumorigenesis associated with VHL. The FDA's approval of Belzutifan in 2021 has been a game-changer, particularly for patients with VHL-associated renal cell carcinoma (RCC), central nervous system hemangioblastomas, and pancreatic neuroendocrine tumors. These therapies are designed to precisely target the molecular drivers of tumor growth, minimizing side effects often seen with traditional chemotherapy, and thereby improving the quality of life for patients

Increased awareness of VHL, a rare genetic disorder, has spurred interest in research and the development of more effective treatment strategies. Research initiatives by academic institutions, hospitals, and pharmaceutical companies are delving deeper into understanding the disease's underlying molecular mechanisms and developing novel therapies. Additionally, the expansion of rare disease awareness campaigns and the establishment of VHL patient registries promote research funding and collaboration across institutions globally. This heightened focus on VHL is fostering a more robust pipeline for drug development and enabling better patient care.

The shift toward personalized medicine is one of the most profound trends in the treatment of VHL. Personalized medicine involves tailoring medical treatment to the individual characteristics of each patient, including genetic profiles. As genetic testing becomes more accessible and affordable, clinicians can develop treatment plans that are specifically suited to each patient's genetic makeup. This ensures that therapies are more effective and less likely to cause adverse reactions. For VHL patients, this might involve using targeted therapies like HIF-2 α inhibitors based on the patient's genetic mutation, thus improving overall survival rates and reducing unnecessary treatments.

The VHL disease market is dominated by both large pharmaceutical companies and biotechnology firms. Major players include:

Merck & Co., with the approval of Belzutifan (Welireg) for treating VHL-associated RCC and other tumors. Novartis Pharmaceuticals, with a focus on developing HIF-2 α inhibitors. Betta Pharmaceuticals and Jiangsu Hansoh Pharmaceutical are both working on oral HIF-2 α inhibitors for the treatment of VHL-related tumors.

These companies are developing innovative therapies and working to expand their market share in both developed and emerging markets.

North America, particularly the United States, is the dominant market for VHL

treatments. The region benefits from advanced healthcare infrastructure, high levels of research funding, and a strong patient population. The presence of numerous clinical trials and research initiatives also contributes to market leadership.

Europe is also a significant market, with countries like the United Kingdom, Germany, and France driving growth. The region's regulatory environment supports the approval of novel therapies, and the increasing adoption of multidisciplinary care for rare diseases is advancing treatment for VHL patients.

Asia-Pacific is experiencing rapid growth in healthcare access and awareness, particularly in countries like China and India. As awareness of VHL increases and healthcare systems improve, there are opportunities for pharmaceutical companies to expand access to treatments and diagnostics.

While healthcare access is more limited in parts of Latin America, the growing recognition of VHL and other rare diseases is opening up opportunities for market expansion. Increasing healthcare investment and improving diagnostics infrastructure in countries like Brazil and Mexico present opportunities for companies to enter the market.

The VHL disease market is positioned for robust growth, driven by advancements in genetic diagnostics, the development of targeted therapies, and increased global research. As the disease is rare, the market remains niche, but the expanding patient population and emerging treatment options are fueling market expansion. Key opportunities lie in gene therapy, the increasing demand for personalized medicine, and the expansion of treatment access in emerging markets. The landscape is competitive, with significant contributions from pharmaceutical companies like Merck, Novartis, and Betta Pharmaceuticals. Regional growth, particularly in Asia-Pacific and Latin America, will further contribute to the overall market development, enhancing patient access to better care and treatment options.

Market Segmentation:

Segmentation 1: by Treatment

Surgery

Targeted Therapies

Radiation Therapy

Segmentation 2: by Clinical Manifestation

Hemangioblastomas

Renal

CNS

Spinal Cord

Renal Cell Carcinoma

Pancreatic Neuroendocrine Tumor

Others

Segmentation 3: by Region

North America

Europe

Asia-Pacific

Rest-of-the-World

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Contents

Executive Summary
Scope and Definition
Market/Product Definition
Key Questions Answered
Analysis and Forecast Note

1. GLOBAL VON-HIPPEL LINDAU DISEASE MARKET: INDUSTRY OUTLOOK

- 1.1 Industry Outlook
 - 1.1.1 Market Introduction
 - 1.1.2 Introduction on Von-Hippel Lindau Disease Market
 - 1.1.2.1 Clinical Manifestation
 - 1.1.2.2 Classification of Von-Hippel Lindau Disease
 - 1.1.2.3 Treatment and Management
 - 1.1.2.3.1 Primary Management
 - 1.1.3 Emerging Therapies
 - 1.1.4 Pipeline Analysis
 - 1.1.5 Regulatory Landscape of Von-Hippel Lindau Disease Market
 - 1.1.5.1 Legal Requirement and Framework in the U.S.
 - 1.1.5.2 Legal Requirement and Framework in the E.U.
 - 1.1.5.3 Legal Requirement and Framework in Japan
 - 1.1.5.4 Legal Requirement and Framework in Rest-of-the-World
- 1.2 Market Dynamics
 - 1.2.1 Impact Analysis
 - 1.2.2 Market Drivers
 - 1.2.3 Market Restraint
 - 1.2.4 Market Opportunities

2. GLOBAL VON-HIPPEL LINDAU DISEASE MARKET (BY TREATMENT), \$MILLION, 2024-2035

- 2.1 Overview
 - 2.1.1 Surgery
 - 2.1.2 Targeted Therapies
 - 2.1.3 Radiation Therapy

3. GLOBAL VON-HIPPEL LINDAU DISEASE MARKET (BY CLINICAL

MANIFESTATION), \$MILLION, 2024-2035

3.1 Overview

3.1.1 Hemangioblastomas

3.1.1.1 Renal

3.1.1.2 CNS

3.1.1.3 Spinal Cord

3.1.2 Renal Cell Carcinoma

3.1.3 Pancreatic Neuroendocrine Tumor

3.1.4 Others

4. GLOBAL VON-HIPPEL LINDAU DISEASE MARKET, BY REGION, \$MILLION, 2024-2035

4.1 Overview

4.2 North America

4.2.1 Business Drivers

4.2.2 Business Challenges

4.2.3 Market Sizing and Forecast

4.2.3.1 North America Von-Hippel Lindau Disease Market, by Country

4.2.3.1.1 U.S.

4.2.3.1.2 Canada

4.3 Europe

4.3.1 Business Drivers

4.3.2 Business Challenges

4.3.2.1 Europe Von-Hippel Lindau Disease Market, by Country

4.3.2.1.1 Germany

4.3.2.1.2 U.K.

4.3.2.1.3 France

4.3.2.1.4 Italy

4.3.2.1.5 Spain

4.3.2.1.6 Rest-of-Europe

4.4 Asia-Pacific

4.4.1 Business Drivers

4.4.2 Business Challenges

4.4.3 Market Sizing and Forecast

4.4.3.1 Asia-Pacific Von-Hippel Lindau Disease Market, by Country

4.4.3.1.1 Japan

4.4.3.1.2 China

- 4.4.3.1.3 India
- 4.4.3.1.4 Australia
- 4.4.3.1.5 South Korea
- 4.4.3.1.6 Rest-of-APAC
- 4.5 Rest-of-the-world
 - 4.5.1 Business Drivers
 - 4.5.2 Business Challenges
 - 4.5.3 Market Sizing and Forecast

5. COMPETITIVE LANDSCAPE AND COMPANY PROFILES

- 5.1 Competitive Landscape
 - 5.1.1 Overview
 - 5.1.2 Key Developments and Strategies
 - 5.1.2.1 Funding Activities
 - 5.1.2.2 Mergers and Acquisitions
 - 5.1.2.3 Regulatory Approvals
 - 5.1.2.4 Partnerships, Collaborations and Business Expansions
 - 5.1.3 Growth-Share Analysis (by Company)
- 5.2 Company Profiles
 - 5.2.1 Merck & Co.
 - 5.2.1.1 Overview
 - 5.2.1.2 Product Portfolio
 - 5.2.1.3 Target Customers
 - 5.2.1.4 Key Personnel
 - 5.2.1.5 Analyst View
 - 5.2.2 Peloton Therapeutics, Inc.
 - 5.2.2.1 Overview
 - 5.2.2.2 Product Portfolio
 - 5.2.2.3 Target Customers
 - 5.2.2.4 Key Personnel
 - 5.2.2.5 Analyst View
 - 5.2.3 Genetech, Inc.
 - 5.2.3.1 Overview
 - 5.2.3.2 Product Portfolio
 - 5.2.3.3 Target Customers
 - 5.2.3.4 Key Personnel
 - 5.2.3.5 Analyst View
 - 5.2.4 Novartis AG

- 5.2.4.1 Overview
- 5.2.4.2 Product Portfolio
- 5.2.4.3 Target Customers
- 5.2.4.4 Key Personnel
- 5.2.4.5 Analyst View
- 5.2.5 Astellas Pharma
 - 5.2.5.1 Overview
 - 5.2.5.2 Product Portfolio
 - 5.2.5.3 Target Customers
 - 5.2.5.4 Key Personnel
 - 5.2.5.5 Analyst View
- 5.2.6 Exelixis
 - 5.2.6.1 Overview
 - 5.2.6.2 Product Portfolio
 - 5.2.6.3 Target Customers
 - 5.2.6.4 Key Personnel
 - 5.2.6.5 Analyst View
- 5.2.7 Amgen, Inc.
 - 5.2.7.1 Overview
 - 5.2.7.2 Product Portfolio
 - 5.2.7.3 Target Customers
 - 5.2.7.4 Key Personnel
 - 5.2.7.5 Analyst View
- 5.2.8 Bayer AG
 - 5.2.8.1 Overview
 - 5.2.8.2 Product Portfolio
 - 5.2.8.3 Target Customers
 - 5.2.8.4 Key Personnel
 - 5.2.8.5 Analyst View

6. RESEARCH METHODOLOGY

List Of Figures

LIST OF FIGURES

Figure: Von-Hippel Lindau Disease Market (by Scenario), \$Million, 2024, 2030, and 2035

Figure: Global Von-Hippel Lindau Disease Market, 2024 and 2035

Figure: Global Von-Hippel Lindau Disease Market Key Trends, Impact Analysis, 2024-2035

Figure: North America Von-Hippel Lindau Disease Market, \$Million, 2024-2035

Figure: Europe Von-Hippel Lindau Disease Market, \$Million, 2024-2035

Figure: Asia-Pacific Von-Hippel Lindau Disease Market, \$Million, 2024-2035

Figure: Rest-of-the-World Von-Hippel Lindau Disease Market, \$Million, 2024-2035

List Of Tables

LIST OF TABLES

Table: Market Snapshot

Table: Market Dynamics

Table: Global Von-Hippel Lindau Disease Market (by Region), \$Million, 2024-2035

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