

# Retinitis Pigmentosa Market Size in the 7MM was ~USD 500 million in 2023, estimated Delvelnsight

Retinitis Pigmentosa Market

DELHI, DELHI, INDIA, June 7, 2024 /EINPresswire.com/ -- Delvelnsight's "Retinitis Pigmentosa Market Insights, Epidemiology, and Market Forecast – 2034" report delivers an in-depth understanding of historical and forecasted epidemiology as well as the Retinitis Pigmentosa market trends in the United States, EU4 (Germany, Spain, Italy, and France) and the United Kingdom, and Japan.



Key Takeaways from the Retinitis Pigmentosa Market Report

- In the US, the total number of prevalent cases of retinitis pigmentosa were  $\sim$ 113,000 in 2023.
- Nonsyndromic retinitis pigmentosa is more prevalent than syndromic retinitis pigmentosa with ~65% cases of total retinitis pigmentosa.
- X-linked retinitis pigmentosa in the US accounted for ~16,000 prevalent cases in 2023.
- Germany has the most cases among EU4 and UK, whereas the UK has the fewest.
- RPE65-linked IRD account for about 3–16% of Leber congenital amaurosis and approximately 0.6–6% of retinitis pigmentosa.
- The leading Retinitis Pigmentosa Companies such as Johnson & Johnson Innovative Medicine, MeiraGTx, Beacon Therapeutics, Nanoscope Therapeutics, Gensight Biologics, 4D Molecular Therapeutics, Coave Therapeutics, Ocugen, Bionic Sight, jCyte, Endogena Therapeutics, ProQR Therapeutics, and Aldeyra Therapeutics and others.
- Promising Retinitis Pigmentosa Therapies such as Botaretigene sparoparvovec, AGTC-501, GS030, 4D 125, CTx PDE6B, OCU 400, EA-2353, Ultevursen, ADX 2191, and others.
- June 2024:- Rolfs Consulting und Verwaltungs-GmbH (RCV)- Participants at risk for a syndromic or a monogenic genetic obesity, incl. participants clinically diagnosed with Bardet-Biedl-Syndrome (BBS).
- May 2024:- PYC Therapeutics- The purpose of this study is to characterize the natural history through temporal systemic evaluation of subjects identified with PRPF31 mutation-associated

retinal dystrophy, also called retinitis pigmentosa type 11, or RP11.

• May 2024:- Janssen Pharmaceutical- Phase 3 Study to Evaluate the Safety and Efficacy of AAV5-hRKp.RPGR for the Treatment of Japanese X-linked Retinitis Pigmentosa Associated With Pathogenic Variants in Retinitis Pigmentosa GTPase Regulator (RPGR). The purpose of the study is to assess the safety and tolerability of bilateral subretinal delivery of adeno-associated virus vector with a serotype 5 capsid human rhodopsin kinase promoter. retinitis pigmentosa guanosine triphosphatase regulator (AAV5-hRKp.RPGR).

Discover which therapies are expected to grab the Retinitis Pigmentosa Market Share @ Retinitis Pigmentosa Market Outlook

## Retinitis Pigmentosa Overview

Retinitis Pigmentosa (RP) is a group of genetic disorders that cause progressive degeneration of the retina, the light-sensitive tissue at the back of the eye. This condition primarily affects the photoreceptor cells, namely rods and cones, leading to a gradual loss of vision. Initially, individuals with RP experience difficulty seeing in low light or at night (nyctalopia) and a reduction in peripheral vision, often described as tunnel vision. As the disease progresses, it can lead to central vision loss, making tasks like reading or recognizing faces challenging. RP is typically inherited and can result from mutations in any of more than 50 different genes. While there is currently no cure, various treatments, including vitamin A supplementation, retinal implants, and gene therapy, are being explored to slow the progression of the disease and improve patients' quality of life.

## Retinitis Pigmentosa Epidemiology Segmentation

- Total Retinitis Pigmentosa Diagnosed Prevalent Population
- Retinitis Pigmentosa Gender-Specific Diagnosed Prevalence
- Retinitis Pigmentosa Type-Specific Diagnosed Prevalence
- Sub-Type Specific Diagnosed Prevalence of Syndromic and Systemic Retinitis Pigmentosa
- Sub-Type Specific Diagnosed Prevalence of Non-Syndromic Retinitis Pigmentosa

Download the report to understand which factors are driving Retinitis Pigmentosa Epidemiology trends @ Retinitis Pigmentosa Epidemiological Insights

# Retinitis Pigmentosa Treatment Landscape

With only one approved therapies like LUXURNA, prompting exploration of off-label and symptomatic treatments, managing this progressive condition involves best supportive care, genetic counseling, and adaptive strategies. The potential therapies in the Retinitis Pigmentosa pipeline, including AGTC-501, Botaretigene sparoparvovec, MCO-010, GS030, ADX-2191, jCell, EA-2353, and others, are advancing through different stages of clinical development, offering promising avenues for effective Retinitis Pigmentosa treatments.

# Retinitis Pigmentosa Marketed Drugs

• LUXTURNA: Sparks Therapeutics (Roche)/Novartis

LUXTURNA (AAV2-hRPE65v2; voretigene neparvovec), known as voretigene neparvovec-rzyl, is a one-time gene therapy for the treatment of patients with vision loss due to a genetic mutation in both copies of the RPE65 gene. It provides a copy of the RPE65 gene to act in place of the mutated RPE65 gene. This working gene can restore vision and improve sight. The drug is administered as a subretinal single injection below the retina in patients who have confirmed RPE65 mutations and viable retinal cells. The drug was developed and commercialized in the US by Spark Therapeutics. In Europe, Novartis is currently marketing LUXTURNA as per a licensing agreement covering the development, registration, and commercialization rights of LUXTURNA in markets outside the US.

#### Retinitis Pigmentosa Emerging Drugs

• Botaretigene sparoparvovec: Johnson & Johnson Innovative Medicine /MeiraGTx Botaretigene sparoparvovec (bota-vec) is designed to treat the most common form of X-linked retinitis pigmentosa (XLRP) caused by mutations in the eye-specific form of the RPGR gene called RPGR open reading frame 15 (RPGR ORF15). Both rods and cones photoreceptors require RPGR ORF15 to function. The Phase I/II clinical trial of bota-vec in adult and pediatric patients is complete, and the Phase III Lumeos clinical trial completed enrollment in 2023. AAV-RPGR has received Fast Track and Orphan Drug designations from the FDA, as well as PRIME, ATMP, and Orphan Medicinal Product designations from the EMA. Currently, the drug is in the Phase III stage of its development for the treatment of X-linked Retinitis Pigmentosa.

### • MCO-010: Nanoscope Therapeutics

Nanoscope's MCO-010 gene therapy utilizes a convenient and well-established intraocular injection for delivery of a gene that encodes for the ambient light-sensitive MCO protein into retinal cells. These therapies are intended to enable retinal cells to detect light so that patients with retinitis pigmentosa or Stargardt disease may see again. MCO-010, is in clinical development for retinitis pigmentosa (RP) and Stargardt disease, which are two rare retinal diseases that cause blindness. MCO-010, recently reported topline results from the RESTORE Phase IIb multicenter, randomized, double-masked, sham-controlled clinical trial in the US for retinitis pigmentosa. The company has also recently completed the Phase II STARLIGHT trial of MCO-010 therapy in patients with Stargardt disease. MCO-010 has received FDA fast track designations and FDA orphan drug designations for both retinitis pigmentosa and Stargardt disease.

#### Retinitis Pigmentosa Drug Market

Optogenetics presents an innovative gene therapy overcoming the limitations of traditional approaches. It operates independently of specific genes and proves effective in late-stage diseases characterized by substantial photoreceptor loss. Retinitis pigmentosa is basically an inherited disease leading to a degeneration of the photoreceptor cells, disrupting the normal physiology of phototransduction. This may be autosomal dominant, autosomal recessive, X-linked, or maternally acquired. Mutations in pre-mRNA splicing cause autosomal dominant retinitis pigmentosa. Autosomal recessive RP is caused when two unaffected individuals who are carriers of the same RP-inducing gene in diallelic form can produce offspring with RP. X-linked RP

is identified with mutations of six genes most commonly occurring at specific loci in the RPGR and RP2 genes. These multiple mutations are produced, causing the degeneration of photoreceptor cells.

#### Retinitis Pigmentosa Market Outlook

As more targetable mutations are discovered, and new targeted Retinitis Pigmentosa drugs are developed, patients and Opthamologists will have an expanding array of Retinitis Pigmentosa treatment options. Given the rapid pace of drug approvals, it is important to pause and ensure sufficient data supports the use of specific agents in the appropriate treatment settings, including adjuvant, consolidation, first-line, or subsequent therapy.

Currently, LUXTURNA (voretigene neparvovec) is the only approved therapy for retinitis pigmentosa and is only authorized for the treatment of a small subpopulation of patients that have the RPE65 mutation. LUXTURNA gene therapy is designed for both adult and pediatric patients experiencing vision loss from Inherited Retinal Disease (IRD). Companies that focus on both adult and pediatric patients are likely to have a larger patient pool. Some key players pursuing this approach include Beacon Therapeutics (AGTC-501), MeiraGTx/Janssen Research & Development (Botaretigene sparoparvovec), 4D Molecular Therapeutics (4D-125), ProQR Therapeutics/Laboratoires Thea (Ultevursen), Coave Therapeutics (CTx-PDE6b), and Ocugen (OCU400).

#### Retinitis Pigmentosa Market Dynamics

The dynamics of the retinitis pigmentosa market are expected to change in the coming years. Gene therapy, available in the market, is utilized to address the effects of defective, disease-causing genes by employing engineered viruses, or viral vectors, to deliver a functional gene version into cells. The rising prevalence of retinitis pigmentosa worldwide has spurred pharmaceutical companies to explore this market, aiming for enhanced revenues through specific research and development strategies.

# Scope of the Retinitis Pigmentosa Market Report

- Coverage- 7MM
- Retinitis Pigmentosa Companies- Johnson & Johnson Innovative Medicine, MeiraGTx, Beacon Therapeutics, Nanoscope Therapeutics, Gensight Biologics, 4D Molecular Therapeutics, Coave Therapeutics, Ocugen, Bionic Sight, jCyte, Endogena Therapeutics, ProQR Therapeutics, and Aldeyra Therapeutics and others.
- Retinitis Pigmentosa Therapies- Botaretigene sparoparvovec, AGTC-501, GS030, 4D 125, CTx PDE6B, OCU 400, EA-2353, Ultevursen, ADX 2191, and others.
- Retinitis Pigmentosa Market Dynamics: Retinitis Pigmentosa Market Drivers and Barriers
- Retinitis Pigmentosa Market Access and Reimbursement, Unmet Needs and Future Perspectives

Discover more about Retinitis Pigmentosa Drugs in development @ <u>Retinitis Pigmentosa Clinical</u> <u>Trials Assessment</u>

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