****Published April 2026***

New gene therapies: retinitis pigmentosa (CAT: VAMVG004)

Product Name	:	**MarketVIEW:** New gene therapies – **retinitis pigmentosa**
Description	:	Global gene therapy commercial assessment
Contents	:	Executive presentation (~150 slides .pdf) + workbook(s) (.xls)
Therapeutic Area	:	Gene therapies
Publication date	:	April 2026
Catalogue No	:	VAMVG004

Background

Retinitis pigmentosa is an inherited retinal dystrophy characterized by progressive, irreversible degeneration of rod and cone photoreceptors in the retina, ultimately leading to severe vision loss and blindness. It is the most common inherited retinal dystrophy, with a broadly consistent global prevalence of roughly 1 in 4,000 individuals, affecting patients across all major markets. The disease is marked by high genetic heterogeneity, with more than 200 disease-causing genes identified to date, and most cases exhibiting autosomal recessive inheritance. Symptoms typically begin in childhood or early adulthood, and the condition steadily worsens over the patient's lifetime, driving substantial clinical, economic, and quality-of-life burden.

Retinitis pigmentosa is currently managed by supportive treatments, focused on low-vision aids, management of complications, and genetic counselling, with no widely available therapies that can halt or reverse photoreceptor degeneration for most genotypes. FDA-approved **Luxturna** *(voretigene neparvovec)* has a very limited role, being indicated only for patients with biallelic RPE65-mediated disease who retain viable retinal cells, and therefore does not address the broader, genetically heterogeneous RP population. This leaves a substantial unmet medical need for disease-modifying interventions capable of preserving or restoring vision over the long term. Gene therapies therefore offer a compelling new treatment paradigm by targeting the root genetic defect at the level of the retina, enabling durable expression of functional proteins after a single administration.

Current **gene therapy** development in retinitis pigmentosa is led by a small group of late-stage candidates spanning early- to end-stage disease and multiple mechanisms. **Ocugen's** OCU400 targets early- to mid-stage RP as a gene-agnostic modifier therapy and is in Phase 3 (liMeliGhT), with rolling BLA submission expected in 2026 and potential FDA approval around **2027**. **Nanoscope's** MCO-010 (vMCO-I) is another gene-agnostic optogenetic approach for late-stage RP, currently in Phase 2b RESTORE. Other notable candidates are **Janssen's** Bota-vec (AAV-RPGR), an AAV-mediated RPGR gene-replacement therapy for X-linked RP; **Beacon Therapeutics**' Laru-zova, an AAV-RPGR gene-replacement candidate for X-linked RP currently in Phase 2/3; and **GenSight** Biologics' GS030, an optogenetic therapy for end-stage RP in ongoing Phase 1/2 development.

This **MarketVIEW** product is a brand-new commercial opportunity assessment focused on the potential of emerging gene therapies for retinitis pigmentosa through to 2040, across 10 major high-income developed markets. It delivers a patient-based,

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interactive forecast model (.xls) and a comprehensive executive presentation (~125 slides). All methodology and key assumptions are clearly documented, and the analysis incorporates pricing and value case studies centred on the potential healthcare costs averted by gene therapy versus the current standard of care. The product also includes an up-to-date review of disease background, epidemiology, the current treatment landscape and the evolving R&D pipeline. This product is ideally suited to organisations seeking a detailed, forward-looking, global forecast for this emerging therapeutic class and is particularly relevant for pharmaceutical and biotech companies, investors, and other stakeholders evaluating the strategic and commercial potential of gene therapies in retinal disorders.

Methodology

VacZine Analytics has closely monitored all significant source material pertaining to retinal diseases and gene therapies in each respective market. Source materials used are academic literature articles, government websites, medical bodies and associations, conference proceedings, social media etc. Previously published research by **VacZine Analytics** in the field of gene therapies has also been utilised.

PRODUCT CONTENTS:

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****This product is a summary presentation (.pdf), an MS-workbook (.xls)

Contents – Summary presentation (.pdf)

PAGES: >125 slides fully referenced/sourced. Available in .pdf form

Contents – MS-Excel workbook (.xls)

United States [stratified by RP disease status]
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Australia
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- 400 References – available upon request

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About VacZine Analytics:

VacZine Analytics is an established strategic research agency based in the United Kingdom. Its aim is to provide disease and commercial analysis for the vaccine industry and help build the case for developing new vaccines and biologics.

For more information, please visit our website www.vacZine-analytics.com

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