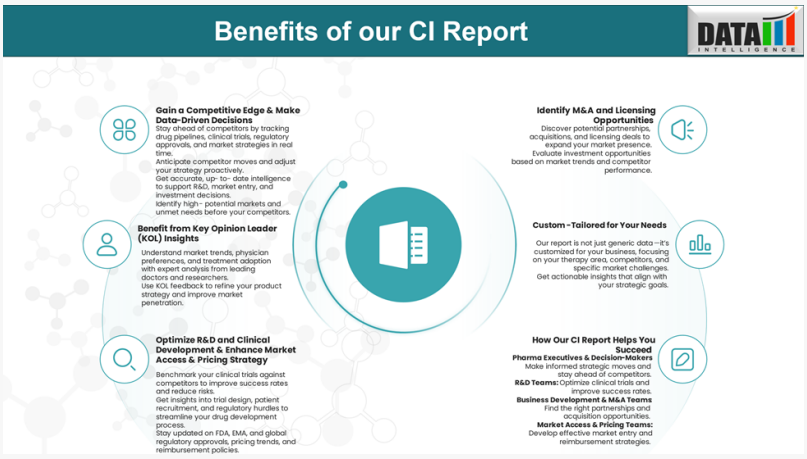
Retinitis Pigmentosa Therapeutics Enter New Era as Gene-Agnostic & Intravitreal Therapies Redefine Competitive Landscape

Retinitis Pigmentosa therapies evolve beyond gene-specific fixes with gene-agnostic, intravitreal, and optogenetic advances reshaping the treatment landscape.



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AUSTIN, TX, UNITED STATES, June 3, 2025 /EINPresswire.com/ -- The treatment paradigm for Retinitis Pigmentosa (RP), a group of rare genetic disorders causing progressive vision loss, is undergoing a transformative shift. According to a new Competitive Intelligence report from DataM Intelligence, emerging therapies are expanding beyond mutation-specific gene fixes to encompass gene-agnostic, optogenetic, RNA, and cell-based modalities—signaling a new frontier in vision restoration and patient accessibility.

> The future of RP therapy lies not just in fixing single genes, but in scalable, non-invasive solutions that restore vision regardless of mutation or disease stage."
>
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Disease Burden and Market Need
RP is the most prevalent inherited retinal dystrophy, affecting over two million people globally. Characterized by early-onset degeneration of rod cells, followed by cone cell deterioration, the disease leads to night blindness, tunnel vision, and eventually complete sight loss. With more than 100 implicated genes, RP remains genetically diverse and highly underserved in terms of therapeutic options.

To date, LUXTURNA (voretigene neparvovec) is the only FDA-approved gene therapy for RP—but it applies to a narrow slice of the patient population (~1–6%) with RPE65 mutations. For the

majority, there is still no disease-modifying treatment, leaving a substantial unmet need.

Pipeline Innovations and Strategic Positioning
The RP pipeline is now populated by a wide array of candidates targeting diverse genetic forms and stages of disease. Leading the charge are late-stage gene therapies such as Botaretigene sparoparvovec (Janssen) for X-linked RP and OCU400 (Ocugen), which uniquely takes a gene-agnostic approach by using modifier gene NR2E3.

Other notable candidates include:
- Sonpiretigene Isteparvovec (Nanoscope): A mutation-independent optogenetic therapy delivered intravitreally, ideal for late-stage patients.
- Ultevursen (Laboratoires Théa/Sepul Bio): An antisense oligonucleotide (ASO) therapy targeting USH2A exon 13 mutations, also delivered intravitreally.
- jCell (jCyte): A cell therapy using allogeneic retinal progenitor cells, designed for patients regardless of genetic background.
- KIO-301 (Kiora Pharmaceuticals): A photoswitch small molecule aimed at reactivating degenerated photoreceptors in low-vision patients.

These therapies collectively represent a dramatic shift in RP treatment: from precision medicine tied to a single mutation to scalable, mutation-independent approaches with non-invasive delivery methods.

Intravitreal Delivery: A Competitive Edge
The evolution from sub-retinal to intravitreal injection marks a practical turning point. Sub-retinal gene therapies like LUXTURNA require surgical intervention, limiting widespread adoption. Intravitreal options like MCO-010, Ultevursen, and jCell are administered in-office, significantly lowering barriers to treatment and aligning with existing ophthalmic care infrastructure.

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Target Opportunity Profile (TOP)
- To compete effectively, new RP therapies must demonstrate:
- Mutation independence or broader genetic applicability
- Practical, minimally invasive delivery (intravitreal preferred)
- Efficacy in mid- to late-stage patients
- Cost-effective scalability
- Long-term safety and durability of effect
- Regulatory acceleration through ODD, RMAT, or PRIME designations

The Target Opportunity Profile (TOP) developed in the report underscores that first-mover therapies offering these advantages could dominate large, currently untapped patient segments.

Strategic Landscape: Key Players and Opportunities
The competitive landscape is heating up. High-intensity competition exists in X-linked RP gene therapies (RPGR), where multiple programs vie for the first approved product. In contrast, optogenetics, RNA therapies, and cell-based treatments remain less crowded, offering a strategic entry point for companies seeking differentiation.

Companies Leading the Charge Include:
- Janssen (Botaretigene sparoparvovec) – poised for regulatory milestones in X-linked RP
- Ocugen (OCU400) – a gene-agnostic platform with RMAT designation
- Nanoscope Therapeutics (MCO-010) – optogenetic therapy targeting late-stage degeneration
- jCyte (jCell) – a scalable cell therapy nearing Phase III trials
- Laboratoires Théa/Sepul Bio (Ultevursen) – first-in-class ASO for USH2A mutations

Conclusion

The Retinitis Pigmentosa therapeutic pipeline is no longer defined by narrow genetic fixes. Instead, it is being reshaped by multimodal innovation, broader patient targeting, and user-friendly delivery systems. With over 2 million affected globally and limited approved options, RP represents one of the most attractive yet underpenetrated markets in rare ophthalmology. Companies that combine clinical efficacy with accessibility, mutation breadth, and scalable delivery will lead the next wave of market disruption.

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