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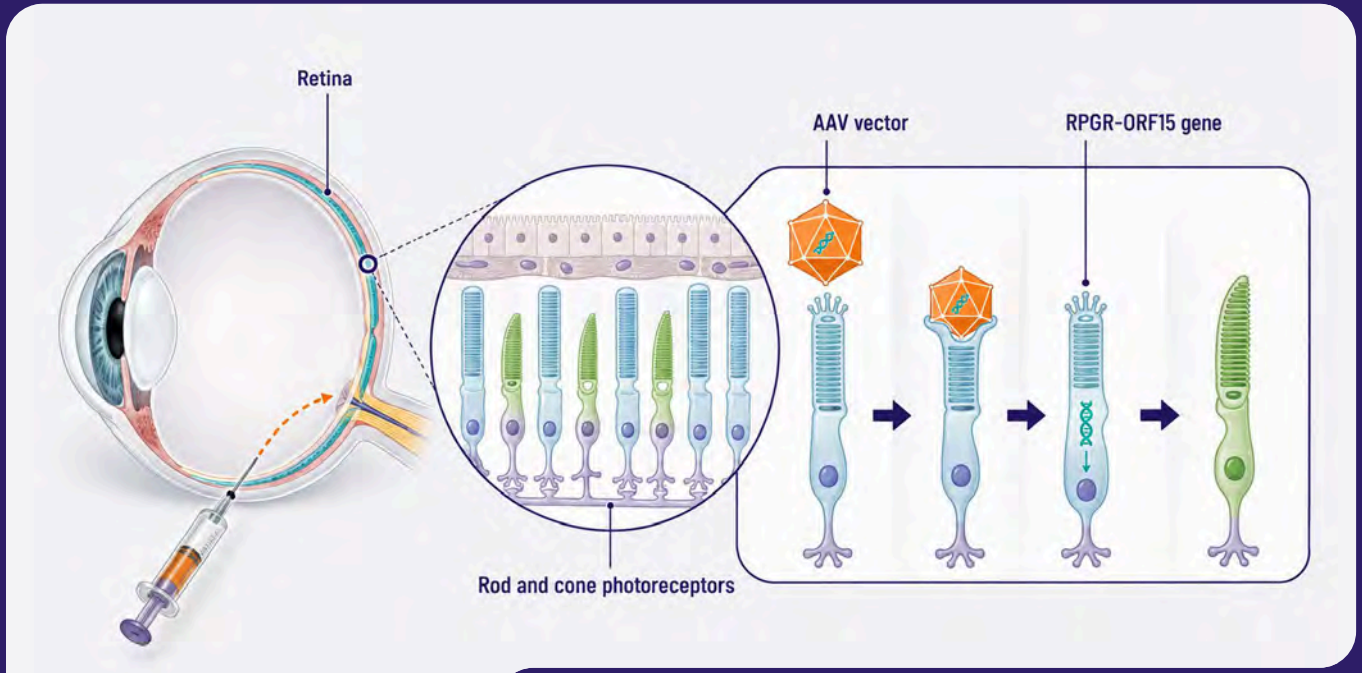
Restoring Sight, One Gene at a Time: Beacon Therapeutics' Pivotal Data Could Mark a Turning Point for an Inherited Retinal Disease

By Dmitrij Hristodorov, PhD, General Partner at Forbion

beacon therapeutics

At least 2.2 billion people worldwide live with some form of vision impairment, and at least 1 billion of those cases are preventable or have yet to be addressed. (1) For people with inherited retinal diseases, the picture is different and often more severe: vision loss is not just a possibility that happens later in life but a near-certainty that can begin in childhood, and for most of these conditions there is still no approved treatment and no way to slow the disease.

That may be about to change for one of them. This September, Beacon Therapeutics, a clinical-stage gene therapy company that Forbion has backed since 2023, expects to report topline data from its pivotal VISTA trial of its lead candidate, laru-zova, in X-linked retinitis pigmentosa (XLRP). (2) A positive result would position laru-zova to become the first approved treatment for this disease and the Company's own earlier-phase clinical data, discussed below, is what grounds that possibility.



Cross-Section of the Human Eye

A disease with no treatment options

XLRP is caused by mutations in the RPGR gene and affects an estimated 3.4 to 4.4 per 100,000 males across the United States, Europe and Australia, which is roughly 1 in 25-29,000. (3) It is a relentless disease: photoreceptors deteriorate gradually, the visual field narrows, and independence erodes well before most patients reach mid-life. Retinitis pigmentosa as a category is the most common inherited cause of blindness, and XLRP is generally its most severe form.

The Science: A One-Time Therapy Designed To Restore Function

Laru-zova uses a modified virus, an adeno-associated viral vector, to deliver a functional copy of the RPGR-ORF15 gene directly to the rod and cone photoreceptors responsible for vision. The intent is to restore the function of these cells by means of a single subretinal injection. Unlike therapies designed only to slow decline, the ambition here is to preserve, and in some patients potentially recover, functional sight.

The earlier-phase evidence is what supports that ambition. Across the Phase 2 SKYLINE and DAWN trials, laru-zova was generally well tolerated, with follow-up now extending to 36 months and safety data out to five years. (4) Both studies showed sustained improvements in low-luminance visual acuity and microperimetry, two accepted measures of visual function. At the Association for Research in Vision and Ophthalmology's (ARVO) 2026 meeting, the company presented a positive 12-month update from DAWN, confirming results first reported at 9 months. (5) The registrational VISTA trial itself enrolled 85 participants aged 12 to 50, across sites in the United States, the United Kingdom and Australia, randomised between two dose levels and an untreated control arm; enrolment completed in June 2025, with topline results expected in Q3 2026. (6) Beacon has also completed dosing in LANDSCAPE, a study of bilateral administration. Laru-zova has Regenerative Medicine Advanced Therapy and Fast Track designations from the U.S. Food and Drug Administration, Priority Medicines designation from the European Medicines Agency, Innovative Licensing and Access Pathway from the UK's Medicines and Healthcare products Regulatory Agency), as well as Orphan Drug Designation from the FDA and EMA. (5)

VISTA Trial (Phase 2/3)

Male patients: 85
Ages: 12–50

- Dose Level 1
- Dose Level 2
- Untreated Control

- North America, United Kingdom, Australia
- Enrolment completed June 2025

Beyond XLRP: a broader pipeline in ocular disease

Beacon's ambitions extend further. Its second program, BTX-001, targets geographic atrophy (GA), an advanced and irreversible stage of dry age-related macular degeneration and one of the leading causes of vision loss in people over 60. (7) BTX-001 is designed for a single intravitreal injection delivering a C5 complement inhibitor, aiming to reduce the repeated-injection burden of existing GA therapies. Clinical entry is planned for later this year.

World-class syndicate support

Beacon's momentum has been matched by investor conviction. The Company has raised approximately $367 million to date, including a $75 million Series C round that closed in January 2026, from Forbion and other blue-chip investors including Syncona, Life Sciences at Goldman Sachs Alternatives, Oxford Science Enterprises, Advent Life Sciences and the Retinal Degeneration Fund (RD Fund), the venture arm of Foundation Fighting Blindness. (8)

> *"At Beacon, we continue to build one of the most promising pipelines in ocular gene therapy. With topline data from our pivotal VISTA trial of laru-zova expected in the third quarter of 2026, we believe this program has the potential to become the first approved therapy for X-linked retinitis pigmentosa, a key breakthrough in ocular gene therapy. Every milestone brings us closer to our mission of delivering lasting impact for people living with blinding retinal diseases."*
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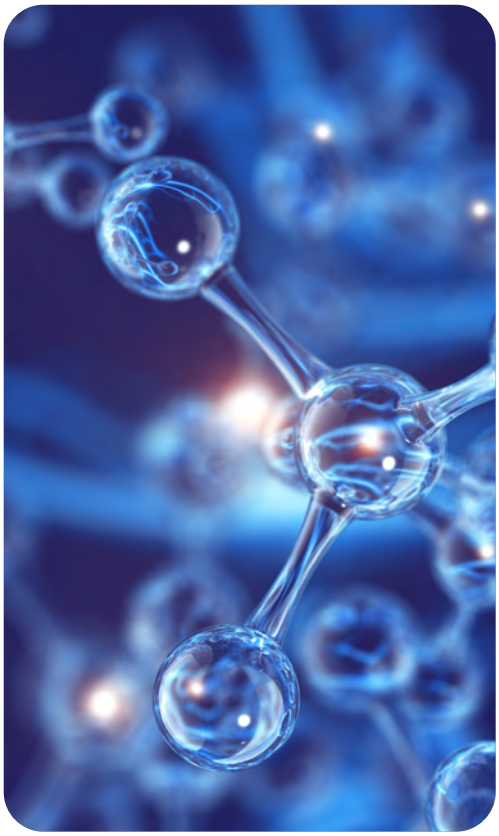
> **- Lance Baldo M.D.**, Chief Executive Officer, Beacon Therapeutics



Approximately

1 in **25,000–29,000** males

Source: Ophthalmic Genetics, 2022

~$367 mln

raised to date

Source: Beacon Therapeutics, January 2026

Why this matters

At Forbion, we invest in science that delivers decisive change for patients. Beacon, with its validated technology platform and experienced leadership team, is approaching a pivotal readout this quarter. That data will not settle the question on its own; regulatory review, and if approved, real-world evidence, still lie ahead. But it is the moment that determines whether laru-zova can move toward becoming the first approved treatment for a disease that currently has none.

References

1. World Health Organization. Blindness and visual impairment fact sheet. who.int/news-room/fact-sheets/detail/blindness-and-visual-impairment

2. Beacon Therapeutics. Completes enrolment in registrational Phase 2/3 VISTA trial of laru-zova, July 2025. beacontx.com

3. Prevalence of RPGR-mutated X-linked retinitis pigmentosa among males. Ophthalmic Genetics, 2022. tandfonline.com/doi/full/10.1080/13816810.2022.2109686

4. Beacon Therapeutics. Positive interim 9+ month DAWN and 36-month SKYLINE data at EURETINA 2025. beacontx.com

5. Beacon Therapeutics. Positive 12-month DAWN update at ARVO 2026 Annual Meeting, May 2026. forbion.com / beacontx.com

6. Ophthalmology Times. Beacon Therapeutics completes enrolment in Phase 2/3 VISTA trial, May 2026. ophthalmologytimes.com

7. Beacon Therapeutics. Geographic Atrophy (GA) secondary to dry AMD — disease and program overview. beacontx.com

8. Beacon Therapeutics. Announces closing of oversubscribed Series C financing for over $75 million, January 2026. beacontx.com

Dmitrij Hristodorov, PhD | General Partner at Forbion

Dmitrij Hristodorov, PhD, is General Partner at Forbion. His current portfolio covers various stages, treatment modalities and disease areas. To date, he serves as a board member for RyCarma, Complement, Seamless, AAVantgarde, Kynexis, Beacon, and Mosanna. He previously served as a board observer of VectorY Therapeutics, as a board observer and R&D committee member of Gyroscope Therapeutics that was acquired for up to $1.5Bn by Novartis in December 2021, as a board director of Rampart Bio, and as a board director of Oxular that was acquired by Regeneron in 2025.