Beacon Therapeutics Reports Positive Topline Data from the Pivotal VISTA Trial of Laru-zova for the Treatment of X-linked Retinitis Pigmentosa (XLRP)

- VISTA met its FDA-endorsed primary endpoint, demonstrating statistically significant low luminance visual acuity (LLVA) responder rates at Month 12 compared to the untreated control group
- A statistically significant proportion of trial participants in both the high and low dose groups achieved an improvement of ≥15 letters in LLVA as compared to no responders in the untreated control group (high dose group responder rate: 31.0%, p=0.0019; low dose group responder rate: 24.1%, p=0.0106)
- VISTA is the first and only pivotal trial in XLRP to achieve its primary endpoint, further building on Beacon's five-year clinical dataset including the prior HORIZON, SKYLINE, and DAWN trials
- Laru-zova demonstrated a favorable safety and tolerability profile, consistent with prior studies
- Based upon these results, Beacon will initiate pre-submission discussions with global regulatory authorities
- Additional data from the VISTA trial will be shared at a late breaking oral presentation at Retina Subspecialty Day during the upcoming AAO Annual Meeting

LONDON and CAMBRIDGE, Mass., September 21, 2026 – Beacon Therapeutics, a leading clinical-stage biotechnology company with a mission to save and restore vision in people with rare and prevalent ocular diseases, today announced positive topline results for its registration-enabling VISTA trial evaluating laruparetigene zovaparvovec (laru-zova) in patients with X-linked retinitis pigmentosa (XLRP).

VISTA (NCT04850118) is a randomized, controlled trial designed to evaluate the efficacy, safety, and tolerability of laru-zova in male patients with XLRP caused by mutations in the retinitis pigmentosa GTPase regulator (*RPGR*) gene. The pivotal trial evaluated 85 male subjects aged 12 to 48 over a 12-month period and included two laru-zova dosing groups: a high dose group of 6.8 E+11 vg/eye and a low dose group of 3.7 E+11 vg/eye. The VISTA trial met its FDA-endorsed primary endpoint with statistical significance. Additionally, laru-zova demonstrated a favorable safety and tolerability profile in the VISTA trial, consistent with prior studies.

“These results represent a landmark moment for the hundreds of thousands of patients worldwide living with XLRP who currently have no treatment options and no way to slow the loss of their sight,” **said Lance Baldo, M.D., Chief Executive Officer of Beacon Therapeutics.** “Today’s data are both statistically significant and clinically meaningful, representing an important milestone for ocular gene therapy and demonstrating the potential for a one-time treatment to change the course of an inherited retinal disease. We are deeply grateful to the patients, families, investigators and advocacy partners who have contributed to this trial, and we now look forward to working closely with regulatory authorities to bring laru-zova to patients as quickly as possible.”

VISTA Topline Results

Efficacy

At Month 12, a statistically significant proportion of participants achieved a ≥15-letter

improvement in LLVA as compared to the untreated control group, with 31.0% of participants in the high dose group (p=0.0019), 24.1% of participants in the low dose group (p=0.0106), and no participants in the untreated control group.

Other secondary endpoints demonstrated supportive positive trends reinforcing consistency of effect across measures of visual function, including improvements in change from baseline in mean macular sensitivity as measured by microperimetry (MP) across the whole grid at Month 12 in treated participants as compared to the untreated control group, and the proportion of participants achieving a ≥10 letter improvement in LLVA.

At Month 12, participants treated with laru-zova demonstrated improvements in mean macular sensitivity as measured by MP across the whole grid compared with the untreated control group. The least-squares mean difference versus untreated control was 1.201 decibels (dB) in the high dose group (p=0.0614) and 1.312 dB in the low dose group (p=0.0405). Additionally, 48.3% of participants in the high dose group (p=0.0002), 58.6% in the low dose group (p<0.0001), and 3.7% in the untreated control group achieved a ≥10-letter improvement in LLVA at Month 12.

Safety and Tolerability

Laru-zova demonstrated a favorable safety and tolerability profile, with predominantly mild to moderate ocular Treatment Emergent Adverse Events (TEAEs) observed that were balanced across treatment groups and largely attributed to the surgical procedure. Laru-zova-related TEAEs were reported in 25% of participants in the high dose group and 38% in the low dose group. Two ocular serious adverse events (SAEs) were observed in the low dose group, both of which were attributed to the surgical procedure.

These results expand the substantial body of evidence supporting the safety and efficacy profile of laru-zova, which now includes five years of clinical experience across 110 treated participants.

“These data culminate years of scientific work and thoughtful clinical trial design and execution,” **said Dr. Robert Sisk, MD, FACS, FASRS, Cincinnati Eye Institute, Professor of Ophthalmology at the University of Cincinnati**. “Beacon selected endpoints that would best capture improvements that matter to patients, particularly their ability to see in low-light conditions, which is one of the most challenging aspects of living with XLRP. Achieving high statistical significance in 15-letter or greater improvement in LLVA demonstrates a clinically meaningful and consistent treatment effect in VISTA, reinforcing confidence in laru-zova’s potential to become a first-in-disease therapy.”

Jason Menzo, Chief Executive Officer of the Foundation Fighting Blindness, added: “For people living with XLRP, this is the news we have been waiting years to hear. XLRP is a relentlessly progressive disease with no approved treatments. This is the first pivotal study of a potential treatment for XLRP to achieve its primary endpoint with statistical significance. Seeing positive study results in endpoints that measure the aspects of vision that matter most in everyday life provides hope for patients and families around the world.”

Next Steps

Based upon these positive topline results, Beacon plans to engage with global regulatory authorities with initiation of a rolling Biologics License Application (BLA) submission planned for later this year.

Oral presentation details at Retina Subspecialty Day, the AAO Annual Meeting, New Orleans, LA, October 9-12, 2026

Presentation title: Subretinal Gene Therapy Laru-zova for X-linked Retinitis Pigmentosa (XLRP): Pivotal VISTA Trial First-time Results
Date: Saturday, October 10 at 4:13pm CST
Presenter: Dr. Robert Sisk, MD, FACS, FASRS, Professor of Ophthalmology, Cincinnati Eye Institute

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About laru-zova

Laruparetigene zovaparvovec (laru-zova) is a potential best-in-class gene therapy currently being evaluated for the treatment of patients with X-linked retinitis pigmentosa (XLRP). Laru-zova has the potential to restore the natural function of both rods and cones in XLRP by delivering a functional copy of the full *RPGR*^{ORF15} gene designed to produce the full-length protein. Laru-zova has Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. Food and Drug Administration (FDA), Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), Innovative Licensing and Access Pathway (ILAP) from the UK's Medicines and Healthcare products Regulatory Agency (MHRA), as well as Orphan Drug Designation (ODD) from the FDA and EMA.

Laru-zova is investigational and has not been approved by FDA for use.

About VISTA

VISTA (NCT04850118) is an ongoing, fully enrolled Phase 2/3 pivotal study evaluating the efficacy, safety and tolerability of laru-zova in two study groups compared to an untreated control group. The study will evaluate the proportion of participants with improvement from baseline in LLVA, mean sensitivity as observed by microperimetry, and additional measures of functional vision.

About the DAWN, SKYLINE, and HORIZON Trials

DAWN (NCT06275620) is an ongoing, fully enrolled, Phase 2, open-label trial of laru-zova in the fellow eye of male participants with XLRP who have previously been treated with an AAV vector-based gene therapy delivering the full-length RPGR protein. The objective of DAWN is to assess two different dose levels of laru-zova for efficacy, safety and tolerability in the target population. DAWN is also evaluating the changes in visual function and functional vision, and is the first trial in the laru-zova clinical development program that is collecting and evaluating low luminance visual acuity (LLVA) data.

SKYLINE (NCT06333249) is an ongoing, fully enrolled, Phase 2, randomized, controlled trial evaluating the safety, efficacy and tolerability of laru-zova in 14 male patients with XLRP caused by mutations in the RPGR gene. The trial's primary endpoint is the proportion of

response by microperimetry between the study and fellow eye at month 12.

HORIZON (NCT03316560) is a completed 5-year Phase 1/2, open-label, dose escalation trial of laru-zova in patients with XLRP due to mutations in the RPGR gene. The trial included 29 male participants, who received a single sub-retinal dose of laru-zova in one eye to obtain initial data about safety and potential efficacy of laru-zova gene therapy.

About X-linked Retinitis Pigmentosa
X-linked retinitis pigmentosa (XLRP) is an inherited retinal disease that predominantly affects males, typically caused by mutations in the retinitis pigmentosa GTPase regulator (RPGR) gene. The mutations, which affect approximately 1 in 25,000 males in the U.S., Europe and Australia, result in progressive photoreceptor loss over time and visual dysfunction beginning in childhood, eventually leading to blindness and impacting quality of life with no approved treatments. It is typically caused by mutations to the retinitis pigmentosa GTPase regulator (RPGR) gene and affects about 10% of the 100,000 retinitis pigmentosa patients in the United States. Laru-zova is a potential best-in-class gene therapy designed to restore the natural function of both rods and cones in XLRP by delivering a functional copy of the full *RPGR*^{ORF15} gene designed to produce the full-length protein.

About Beacon Therapeutics
Beacon Therapeutics is a clinical-stage biotechnology company dedicated to saving and restoring sight for people living with rare and prevalent ocular diseases. The Company is harnessing the transformative power of gene therapy to deliver the most meaningful outcomes for severe ocular diseases. Beacon's pipeline currently targets devastating blinding retinal diseases such as X-linked retinitis pigmentosa (XLRP) and geographic atrophy (GA) secondary to dry age-related macular degeneration (AMD).

Beacon Therapeutics' investors include Advent Life Sciences, Forbion, Life Sciences at Goldman Sachs Alternatives, Oxford Science Enterprises, the Gund Vision Fund, Syncona Limited, and TCGX, among others. Learn more about Beacon Therapeutics at beacontx.com and follow on LinkedIn for more updates.

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