RNS Number : 0497Y
Syncona Limited
04 September 2025

4 September 2025

Syncona Limited ("Syncona")

Beacon announces positive interim data for Phase 2 studies in XLRP

Syncona Ltd, a leading life science investor, today notes that its portfolio company Beacon Therapeutics ("Beacon") announced new results from two Phase 2 trials, SKYLINE and DAWN, evaluating the company's lead programme, laru-zova, in patients with X-linked retinitis pigmentosa (XLRP).

The data demonstrated that laru-zova was generally well-tolerated by SKYLINE participants through month 36 and DAWN participants at 9 months or beyond and showed sustained improvements across several key measures of visual function, including low luminance visual acuity and microperimetry. The results were presented at the EURETINA 2025 Conference being held 4-7 September 2025, in Paris, France.

Beacon is also evaluating low luminance visual acuity as the primary endpoint in its ongoing pivotal VISTA trial of laru-zova for the treatment of XLRP. Beacon recently completed enrolment of this trial and expects to report topline data in the second half of calendar year 2026, which the Syncona Investment Management Limited team view as a potential key value inflection point for the company.

Chris Hollowood, Chief Executive Officer of Syncona Investment Management Limited, said: "We are pleased to see further data from Beacon's DAWN and SKYLINE clinical trials, which underline the potential of its gene therapy to have a profound impact on patients living with a devastating blinding condition. We look forward to continuing to work closely with the company as it progresses through its pivotal study."

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Forward-looking statements - this announcement contains certain forward-looking statements with respect to the portfolio of investments of Syncona Limited. These statements and forecasts involve risk and uncertainty because they relate to events and depend upon circumstances that may or may not occur in the future. There are a number of factors that could cause actual results or developments to differ materially from those expressed or implied by these forward-looking statements. In particular, many companies in the Syncona Limited portfolio are conducting scientific research and clinical trials where the outcome is inherently uncertain and there is significant risk of negative results or adverse events arising. In addition, many companies in the Syncona Limited portfolio have yet to commercialise a product and their ability to do so may be affected by operational, commercial and other risks.

Syncona Limited seeks to achieve returns over the long term. Investors should seek to ensure they understand the risks and opportunities of an investment in Syncona Limited, including the information in our published documentation, before investing.

Beacon Therapeutics Announces Positive Interim 9+ Month Results from DAWN Trial and 36-Month Phase 2 SKYLINE Trial Data for Laru-zova in Patients with X-linked Retinitis Pigmentosa (XLRP) at EURETINA 2025

Data showed sustained improvements across several key measures of visual function, including low luminance visual acuity and microperimetry

Laru-zova was generally well-tolerated by SKYLINE participants through month 36 and DAWN participants at 9 months or beyond

LONDON and CAMBRIDGE, Mass., Sept. 4, 2025 - Beacon Therapeutics Holdings Limited ('Beacon Therapeutics' or 'the Company'), a leading clinical-stage biotechnology company with a mission to save and restore vision in people with rare and prevalent ocular diseases, today announced new results from two Phase 2 trials, SKYLINE and DAWN, evaluating the Company's lead program, laru-zova, in patients with XLRP. The data demonstrated that laru-zova was generally well-tolerated by SKYLINE participants through month 36 and DAWN participants at 9 months or beyond and showed sustained improvements across several key measures of visual function, including low luminance visual acuity and microperimetry. The results were presented at the EURETINA 2025 Conference being held September 4-7, 2025, in Paris, France.

"We are pleased to be sharing key data from our DAWN and SKYLINE trials, building on one of the most significant bodies of evidence for a gene therapy in ocular diseases," said **Daniel Chung, D.O., M.A., Chief Medical Officer of Beacon Therapeutics**. "These new data updates reinforce our belief in the potential for laru-zova to be a meaningful treatment option for people living with XLRP. We look forward to continuing to advance laru-zova through clinical development, while engaging with regulators and the patient community."

XLRP is an inherited retinal disease that often leads to blindness, with no available treatment options. It is typically caused by mutations to the retinitis pigmentosa GTPase regulator (*RPGR*) gene, affecting approximately 1 in 25,000 males in the U.S., Europe and Australia. 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 $RPGR^{ORF15}$ gene designed to produce the full-length protein.

Key DAWN data highlights include:

- Data continued to show early improvements in low luminance visual acuity (LLVA) and early and sustained improvements in mean sensitivity in study eyes, as observed by microperimetry, representing enhanced visual function in participants evaluated at month 9 or beyond.
- Laru-zova continued to be well-tolerated by all participants evaluated at month 9 or beyond.

Key SKYLINE data highlights include:

- Participants who received the high dose of laru-zova showed durable improvements in retinal sensitivity through month 36, as observed by microperimetry.
- There was a greater response rate in the high-dose study eyes compared to the low-dose group or untreated fellow eye.
- Laru-zova continued to be well-tolerated by participants in both low- and high-dose groups through month 36.

Beacon is also evaluating LLVA as the primary endpoint in its ongoing pivotal VISTA trial of laru-zova for the treatment of XLRP. Beacon recently completed enrollment of this trial and expects to report topline data in the second half of 2026.

Presentation details:

Presentation title: Subretinal gene therapy laru-zova for X-linked retinitis pigmentosa (XLRP): Phase 2 DAWN trial, preliminary month 9+ results
Date: Thursday, September 4, 2025
Presenter: Rajiv Anand, M.D., FRCS, FRCOphth, Texas Retina Associates and Retina Foundation of the Southwest

Presentation title: Subretinal laru-zova gene therapy for XLRP: 36-month results of the randomized, controlled, multicenter Phase 2 SKYLINE trial
Date: Thursday, September 4, 2025
Presenter: Paul Yang, M.D., Ph.D. Chief, Paul H. Casey Ophthalmic Genetics Division, Casey Eye Institute, OHSU

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About laru-zova
Laru-zova (laruparetigene zovaparvovec) is a potential best-in-class gene therapy currently being investigated 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 $RPGR^{ORF15}$ 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 the DAWN and SKYLINE Studies
DAWN (NCT06275620) is an ongoing, fully enrolled, Phase 2, open-label study 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 is an ongoing, fully enrolled, Phase 2, randomized, controlled study evaluating the safety, efficacy and tolerability of laru-zova in 14 male patients with XLRP caused by mutations in the *RPGR* gene. The study's primary endpoint is the proportion of response by microperimetry between the study and fellow eye at Month 12. NCT06333249.

About XLRP
X-linked retinitis pigmentosa (XLRP) is an inherited retinal disease that predominantly affects males, typically caused by mutations in the *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.

About Beacon Therapeutics
Beacon Therapeutics is a clinical-stage biotechnology company with a mission to save and restore the sight and improve the lives of people living with rare and prevalent ocular diseases to help them to live life to the fullest. The Company is harnessing the transformative power of gene therapy where they can deliver the most meaningful

Company is harnessing the transformative power of gene therapy where they can deliver the most meaningful outcomes for severe ocular diseases. Beacon's pipeline currently targets high-impact blinding retinal diseases such as X-linked retinitis pigmentosa (XLRP) and geographic atrophy.

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

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