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Beacon Therapeutics announced positive data from Phase II DAWN trial

Syncona Ltd, ("Syncona" or the "Company") a leading life science investor focused on creating, building and scaling global leaders in life science, notes that its portfolio company Beacon Therapeutics ("Beacon") announced six-month interim safety and efficacy results from its Phase II DAWN trial of the company's lead programme, laru-zova (laruparetigene zovaparvovec), in patients with X-linked retinitis pigmentosa (XLRP) at the Association for Research in Vision and Ophthalmology (ARVO) 2025 Annual Meeting. The publishing of this data is a capital access milestone[1] for Beacon.

Key highlights from the presentation include:

- Patients in the trial showed improvements across several key visual function measures, demonstrating laru-zova's potential to enhance vision in patients with XLRP
  - Data demonstrated early improvements in low luminance visual acuity (LLVA), an important measure of visual function, with greater two and three line improvements in the study eyes compared to previously treated fellow eyes in participants evaluated at month six or beyond.
  - Data also showed early and sustained improvements in mean sensitivity in study eyes, as observed by microperimetry, indicating enhanced visual function in participants evaluated at month six or beyond.
- Laru-zova was generally well-tolerated by patients in the DAWN trial at six months
  - Ocular treatment-emergent adverse events were generally non-serious and mild or moderate in severity, with a majority related to surgical procedures and steroids required by the protocol that have since resolved
  - There were no suspected unexpected serious adverse reactions, retinal detachments or endophthalmitis (inflammation inside the eye) reported

Elisa Petris, Partner of Syncona Investment Management Limited and Board Director of Beacon Therapeutics, said: "We are really pleased with the progress at Beacon. It is fantastic to see continued visual improvements in patients evaluated at the six-month time point in this data update. We are optimistic as the company progresses enrolment for its Phase II/III pivotal study and look forward to seeing data in this crucial programme. We see a really differentiated opportunity with this therapy to transform the lives of patients with a devastating retinal blinding condition."

Beacon's announcement is copied below and can be accessed at the company's website at beacontx.com.

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About Syncona

Syncona's purpose is to invest to extend and enhance human life. We do this by creating, building and scaling

companies to deliver transformational treatments to patients in areas of high unmet need.

We aim to build and maintain a diversified portfolio of 20-25 globally leading life science businesses, across development stage, modality and therapeutic area, for the benefit of all our stakeholders. We focus on developing treatments that deliver patient impact by working in close partnership with world-class academic founders and experienced management teams. Our balance sheet underpins our strategy, enabling us to take a long-term view as we look to improve the lives of patients with no or poor treatment options, build sustainable life science companies and deliver strong risk-adjusted returns to shareholders.

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.

About Capital Access Milestones

A capital access milestone is a de-risking event for a portfolio company that is expected to enable access to capital, which underpins progression towards a company's next milestone. It is less likely that a capital access milestone (relative to a key value inflection point) will drive significant NAV growth for Syncona, for example by increasing the possibility of a realisation event, such as M&A.

Beacon Therapeutics Announces Positive Phase 2 Interim 6-Month Data from DAWN Trial of Laru-zova in Patients with X-linked Retinitis Pigmentosa (XLRP) at ARVO 2025

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

Laru-zova was generally well-tolerated by DAWN participants at 6 months

LONDON and CAMBRIDGE, Mass., May 6, 2025 - Beacon Therapeutics Holdings Limited ('Beacon Therapeutics' or 'the Company'), a leading clinical-stage biotechnology company with a mission to save and restore the vision of patients with blinding retinal diseases, today announced 6-month interim safety and efficacy results from the Phase 2 DAWN trial of the Company's lead program, laru-zova (laruparetigene zovaparvovec), in patients with X-linked retinitis pigmentosa (XLRP) at the Association for Research in Vision and Ophthalmology (ARVO) 2025 Annual Meeting being held May 4-8, 2025 in Salt Lake City. Laru-zova was generally well-tolerated by all DAWN participants evaluated at 6 months or beyond and initial data showed promising improvements in visual function across several key measures.

"Over the past five years we have built a compelling body of safety and efficacy data on laru-zova across three different clinical studies," said **Lance Baldo, M.D., Chief Executive Officer of Beacon Therapeutics.** "We are pleased to be sharing the 6-month data update from the DAWN Phase 2 study that continues to demonstrate laru-zova's potential to enhance vision in patients with XLRP, including improvements in multiple measures of visual function. We look forward to continuing the advancement of this exciting novel treatment option for patients suffering from XLRP."

XLRP is an inherited retinal disease often caused by mutations to the RPGR gene, affecting 1 in 25,000 males in the U.S., Europe and Australia. The disease often leads to blindness by middle age, with no available treatment options. 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 using a well-established vector with a proprietary capsid designed for high transduction of photoreceptors, and a codon-optimized gene to produce the full-length protein.

Key data highlights include:

- Ocular treatment-emergent adverse events (TEAEs) were generally non-serious and mild or moderate in severity, with a majority related to surgical procedures and steroids required by the protocol that have since resolved. There were no suspected unexpected serious adverse reactions, retinal detachments or endophthalmitis reported.
- Data demonstrated early improvements in low luminance visual acuity (LLVA), an important measure of visual function, with a greater number of two and three line improvements in the study eyes compared to previously treated fellow eyes in participants evaluated at month 6 or beyond.
- Data also showed early and sustained improvements in mean sensitivity in study eyes, as observed by microperimetry, indicating enhanced visual function in participants evaluated at month 6 or beyond.

DAWN is an open-label study of laru-zova in participants with XLRP who have previously been treated with a full-length AAV vector-based gene therapy targeting the RPGR protein. The study aims to assess two dose levels of laru-zova for efficacy, safety and tolerability in the untreated eye of participants who previously received gene therapy for XLRP.

The Company continues to enroll patients for its pivotal Phase 2/3 VISTA trial of laru-zova for patients with XLRP.

Presentation Title: Subretinal gene therapy laru-zova (AGTC-501) for X-linked retinitis pigmentosa (XLRP): Phase 2 DAWN preliminary month 6+ results

Presenting Author: Mark Pennesi, M.D., Ph.D., FARVO, Director, Ophthalmic Genetics at the Retina Foundation in Dallas, Texas; Professor of Ophthalmology and Professor of Molecular and Medical Genetics, and Chief of the Paul H. Casey Ophthalmic Genetics Division at the Casey Eye Institute, Oregon Health and Science University in Portland, Oregon

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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 VISTA Studies

DAWN (NCT06275620) is a 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.

VISTA (NCT04850118) is a Phase 2/3, randomized, controlled, masked, multi-center 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 a 15 or more letter increase from baseline in LLVA and additional measures of functional vision. The VISTA study is currently enrolling.

About XLRP

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.

About Beacon Therapeutics

Beacon Therapeutics is a clinical-stage biotechnology company with a mission to save and restore the vision of patients with blinding retinal diseases. The Company has an established scientific foundation that includes a late-stage clinical development candidate to treat XLRP and two preclinical programs targeting dry age-related macular degeneration (AMD) and an inherited cone-rod dystrophy (CRD).

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

[1] Defined in Notes to Editors

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