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Syncona Limited

Beacon announces positive three-month data from Phase II DAWN trial of laru-zova (AGTC-501) in XLRP

Syncona Ltd, ("Syncona") a leading life science investor focused on creating, building and scaling global leaders in life science, today notes that its portfolio company Beacon Therapeutics ("Beacon") announced the presentation of three-month interim safety and efficacy data from the Phase II DAWN trial of its lead asset, laru-zova (AGTC-501), in patients with X-linked retinitis pigmentosa (XLRP), at the FLORetina-ICOOR Meeting 2024 in Florence, Italy. The publishing of this data is a key value inflection point for Beacon[1].

The DAWN study treats patients with XLRP who have previously been treated with an AAV gene therapy in their other eye. The purpose of DAWN is to assess two different doses of laru-zova for efficacy, safety, and tolerability in the untreated eye of participants who previously received gene therapy for XLRP.

Key highlights from the announcement include:

- The three-month data demonstrate that laru-zova has been well-tolerated by all participants
- The data also show promising early improvements in low luminance visual acuity (LLVA), which is a critical measure of visual function and a marker of early disease progression
- The data support the ongoing development of laru-zova in XLRP, a severe, aggressive, inherited retinal disease that impacts boys and young men and often leads to blindness by middle age, with no treatment options available

Beacon Therapeutics is currently enrolling for its Phase II/III pivotal VISTA trial of laru-zova in XLRP, with a data readout expected in CY2026, a key value inflection point for the company.

Chris Hollowood, Chief Executive Officer of Syncona Investment Management Limited and Board Director of Beacon Therapeutics, said: "Building on the recent 24-month data from the Phase II SKYLINE trial, these positive results from DAWN further demonstrate the potential of Beacon's laru-zova programme. The early visual improvements observed reinforce the compelling efficacy data shown to date and offer further hope to XLRP patients. We believe that laru-zova could be a life-changing treatment for these patients and look forward to further data, including from the Phase II/III VISTA trial, which will be used to support regulatory approval."

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 Key Value Inflection Points

A key value inflection point is a material de-risking event for a portfolio company that has the potential to drive significant NAV growth for Syncona, for example by increasing the possibility of a realisation event, such as M&A. These milestones can also enable companies to access significant capital including through financings and IPOs, which may take place at valuation uplifts and underpin progression to a subsequent key value inflection point which has the potential to drive greater value. M&A or capital access is unlikely to occur immediately following a key value inflection point.

Beacon Therapeutics Announces Positive 3-Month Data from Phase 2 DAWN Trial of laru-zova (AGTC-501) in Patients with X-Linked Retinitis Pigmentosa (XLRP)

- To date, laru-zova has been well-tolerated by all participants in the Phase 2 DAWN study.
- Data show promising early improvements in low luminance visual acuity (LLVA), a critical measure of visual function.
- Data build confidence in laru-zova as a potential treatment for patients with XLRP.
- Pivotal Phase 2/3 VISTA trial for laru-zova in XLRP is currently enrolling.

London, UK, Cambridge, MA, 6 December 2024 - Beacon Therapeutics Holdings Limited ('Beacon Therapeutics' or 'the Company'), a leading ophthalmic gene therapy company with a purpose to save and restore the vision of patients with blinding retinal diseases, today announced the presentation of 3-month interim safety and efficacy results of the Phase 2 DAWN trial in patients with XLRP at the FLORetina-ICOOR Meeting 2024 in Florence, Italy.

Key presentation highlights:

- The three-month data show that laru-zova has been well-tolerated by all participants.
- No study agent-related treatment emergent adverse events (TEAEs) were reported, including no ocular inflammatory adverse events.
- Data also show promising early improvements in LLVA, a critical measure of visual function.
- The benefit-risk profile supports on-going clinical development for the treatment of patients with XLRP caused by RPGR mutations.

DAWN is a non-randomized, 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 purpose of DAWN is to assess two different doses of laru-zova for efficacy, safety and tolerability in the untreated eye of participants who previously received gene therapy for XLRP.

XLRP is a severe, aggressive, inherited retinal disease that often leads to blindness by middle age, with no treatment options available. XLRP primarily affects young males with an estimated prevalence of 1 in 25,000 males in US, Europe and Australia having XLRP with RPGR mutations. Laru-zova expresses the full length RPGR protein and is therefore expected to address the entirety of photoreceptor damage caused by XLRP, including both rod and cone loss, representing a potential best-in-class treatment for progressive vision loss in patients with XLRP.
Lance Baldo, MD, chief executive officer of Beacon Therapeutics, stated, "We are encouraged by the early results from the Phase 2 DAWN study. The strong safety profile observed to date is complemented by promising early

improvements in low luminance visual acuity - a meaningful and functional measure of vision in patients with XLRP. These data not only support the ongoing pivotal VISTA study, but also strengthen our commitment to this opportunity to bring hope to patients and families affected by this devastating disease."
Beacon Therapeutics is also enrolling patients for its pivotal Phase 2/3 VISTA trial of laru-zova as it develops this potential treatment for patients with XLRP.

Presentations -

- Subretinal Gene Therapy laru-zova (AGTC-501) for X-Linked Retinitis Pigmentosa (XLRP) Phase 2 Multicenter Study (DAWN): Preliminary Results

- RPGR gene therapy and the Beacon clinical trials: Beacon Therapeutics Subretinal Gene Therapy laru-zova (AGTC-501) for X-Linked Retinitis Pigmentosa (XLRP)

Presenting Author - Professor Paulo Eduardo Stanga, Founder and Chief Medical Officer, The Retina Clinic London

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About Beacon Therapeutics
Beacon Therapeutics is an ophthalmic gene therapy company founded in 2023 to save and restore the vision of patients with a range of prevalent and rare retinal diseases that result in blindness.

The Company has an established scientific foundation that combines a late-stage development candidate to treat X-linked retinitis pigmentosa (XLRP) and two preclinical programs, one targeting dry age-related macular degeneration (AMD) and another targeting an inherited cone-rod dystrophy (CRD). Beacon Therapeutics also has access to a target generation technology platform that will identify, screen, and search secreted proteins in the ophthalmology space.

Lead development candidate laru-zova (AGTC-501), is a gene therapy program currently being investigated for the treatment of XLRP, an inherited monogenic recessive disorder that causes progressive vision loss, primarily in boys and young men. XLRP is predominantly caused by mutations in the retinitis pigmentosa GTPase regulator (RPGR) gene. Laru-zova expresses the full length RPGR protein, thereby addressing the full complement of photoreceptor damage caused by XLRP, including both rod and cone loss.

Beacon is supported by funds from Syncona Limited, Forbion, Oxford Science Enterprises, TCGX, Advent Life Sciences and additional investors.

Find out more about Beacon Therapeutics at beacontx.com.

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[1] See definition of key value inflection points in the note section

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