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Beacon announces positive 24-month data from Phase II trial of AGTC-501 in XLRP

Syncona Ltd, ("Syncona" or the "Company") 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 24-month interim safety and efficacy data from the Phase II SKYLINE trial of its lead asset, AGTC-501, in patients with X-linked retinitis pigmentosa (XLRP), at the American Academy of Ophthalmology's Annual Meeting in Chicago, Illinois. The publishing of this data is a potential key value inflection point for Beacon.

Key highlights from the presentation include:

- 24-month analysis indicated AGTC-501 was generally well tolerated with no clinically significant safety events associated with treatment
- Data demonstrates the durable efficacy profile of AGTC-501 with the higher dose cohort showing a 57% response rate in the 24-month analysis of retinal sensitivity, the primary endpoint for the trial[1]
- Benefit-risk profile supports on-going clinical development for AGTC-501 for the treatment of patients with XLRP, a severe, aggressive, inherited retinal disease that often leads to blindness by middle age with no treatment options available
- Beacon continues to enrol patients in its pivotal Phase II/III VISTA trial and its Phase II DAWN trial for AGTC-501 in XLRP

Chris Hollowood, Chief Executive Officer of Syncona Investment Management Limited and Board Director of Beacon Therapeutics, said: "This data supports our thesis that AGTC-501 can be a potentially life-changing treatment for patients suffering from XLRP. The continued efficacy of the therapy at 24-months post-dosing underlines the potential of a one-time gene therapy for this condition, with the data also showing a strong safety profile. The delivery of this milestone is a potential key value inflection point for Beacon, and we look forward to seeing upcoming data from the Phase II DAWN trial and further progress in the Phase II/III VISTA trial, with data from both of these trials along with SKYLINE supporting the company's regulatory strategies in the US and EU."

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.

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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.

Beacon Therapeutics Announces Positive 24-Month Data from Phase 2 SKYLINE Trial of AGTC-501 in Patients with X-Linked Retinitis Pigmentosa

- 24-month analysis indicated AGTC-501 was generally safe and well-tolerated, with no clinically significant safety events associated with treatment
- Data demonstrates the durable profile of AGTC-501, with the higher dose cohort showing a 57% response rate in the 24-month analysis of retinal sensitivity, the primary endpoint for the trial
- Benefit-risk profile supports on-going clinical development for the treatment of patients with X-Linked Retinitis Pigmentosa (XLRP) caused by retinitis pigmentosa GTPase regulator (RPGR) gene mutations
- Pivotal Phase 2/3 VISTA and open-label Phase 2 DAWN trials for AGTC-501 in XLRP are currently enrolling

London, UK, Cambridge, MA, 15 October 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 24-month interim safety and efficacy results of the Phase 2 SKYLINE trial in patients with XLRP at the American Academy of Ophthalmology's Annual Meeting in Chicago, Illinois.

The 24-month data showed a response rate of 57% (4/7) in study eyes treated with a high dose (6.8 E+11 vg/eye) of AGTC-501, defined as a patient who has an improvement in retinal sensitivity as assessed by microperimetry of at least 7 decibels (dB) in at least 5 loci. Patients in the high-dose cohort also showed a robust improvement in mean retinal sensitivity. Study eyes treated with a low dose (7.5 E+10 vg/eye) of AGTC-501 showed a response rate of 25% (1/4). There were no clinically significant safety events associated with AGTC-501 treatment in this trial, and any treatment-emergent adverse events were mostly non-serious and mild to moderate in severity.

XLRP is a severe, aggressive, inherited retinal disease that often leads to blindness by middle age, with no treatment options available. XLRP primarily affects an estimated 3.4 - 4.4 per 100,000 young males with RPGR mutations in US, Europe and Australia. AGTC-501 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, making it uniquely well-suited to improve the lives of patients with XLRP as a potential treatment.

Lance Baldo, MD, Chief Executive Officer of Beacon Therapeutics, stated, "Our Phase 2 SKYLINE 24-month data reinforces AGTC-501's favorable safety profile and robust improvements in mean retinal sensitivity. We will continue to assess the long-term safety and durability of AGTC-501 but are encouraged by the results we've seen in the SKYLINE trial to date."

Beacon Therapeutics is also enrolling its pivotal Phase 2/3 VISTA and open-label Phase 2 DAWN trials as the Company progresses toward the development of a treatment for XLRP.

Poster - Subretinal AGTC-501 Gene Therapy for XLRP: 24-Month Interim Results of the Phase 2 SKYLINE Trial.

Presenting Author - Robert Sisk, MD, FACS, FASRS, Associate Professor of Ophthalmology at the University of Cincinnati; Vitreoretinal Surgeon and partner at Cincinnati Eye Institute; Director of Pediatric Retinal Surgery and Director of Ophthalmic Genetics at Cincinnati Children's Hospital.

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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), as well as 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 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. AGTC-501 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] At the 12-month cut off the response rate was 63% (five out of eight patients). The 24-month analysis shows responses from four out of seven patients with one patient missing the scheduled 24-month visit.