Beacon Therapeutics Presents 36-Month Interim Results from Phase I/2 HORIZON Trial of AGTC-501 in Patients with XLRP

London, UK, and Cambridge, MA, September 19, 2024 – Beacon Therapeutics Holdings Limited ('Beacon' 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 presented 36-month interim results from its Phase I/2 HORIZON trial of its lead asset, AGTC-501, in patients with X-linked retinitis pigmentosa (XLRP) at the 24th EURETINA Congress held in Barcelona, Spain.

Key presentation highlights included:

- AGTC-501 was reported to be generally safe and well-tolerated in the 29 patients enrolled with no clinically significant safety events related to the therapy.
- Data also demonstrated that a difference in visual function between the treated and untreated eyes was still observed at month 36.
- The benefit-risk profile of AGTC-501 supports ongoing clinical development for the treatment of patients with XLRP caused by RPGR mutations.

Lance Baldo, MD, Chief Executive Officer of Beacon, stated, "This emerging longer-term data is another clinical validation of the safety of AGTC-501 for the treatment of XLRP. We look forward to achieving several upcoming clinical milestones, including 24-month data from the Phase 2 SKYLINE trial in XLRP later this year, and continued enrollment into our open-label Phase 2 DAWN trial and Phase 2/3 VISTA trial."

HORIZON is a Phase 1/2, open-label, dose-escalation study of patients with XLRP treated with subretinal AGTC-501, which has completed enrollment of 29 male participants and all participants are in long-term follow-up.

Presentation – Subretinal Gene Therapy Drug AGTC-501 for X-Linked Retinitis Pigmentosa (XLRP) Phase 1/2 Multicenter Study (HORIZON): 36-Month Interim Results

Presenter – Paul Yang, MD, PhD, Chief, Paul H. Casey Ophthalmic Genetics Division, Casey Eye Institute, OHSU

The presentation took place on Thursday, September 19th at 15:30 CEST.

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 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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