Moving to market

We are pleased with the positive clinical data Beacon has reported this year and look forward to the upcoming 24-month data from the Phase II SKYLINE trial, which we expect will be a key value inflection point for the business."

Elisa Petris
BEACON BOARD DIRECTOR AND SIML LEAD PARTNER

Progressing therapies through late-stage development

MATURING DATA SUPPORTING POTENTIAL OF CLINICAL PROGRAMMES

During the year Beacon published positive 12-month data from its Phase II SKYLINE trial of AGTC-501 in X-Linked Retinitis Pigmentosa (XLRP), with the data demonstrating a favourable efficacy and safety profile with improvements in visual function amongst treated patients. The positive data supported further investigation of AGTC-501 in XLRP, with the subsequent initiation of the Phase II DAWN trial and the post-period announcement of the initiation of the registrational Phase II/III VISTA trial[1]. The company expects to announce 24-month durability data from the SKYLINE trial in H2 CY2024, with data readouts to follow from DAWN and VISTA in CY2025 and CY2026, respectively.

ALIGNING MANUFACTURING CAPABILITIES WITH COMMERCIAL LAUNCH STRATEGY

In April 2024, Ascend Advanced Therapies announced the acquisition of Beacon's chemistry, manufacturing and controls (CMC) team and good manufacturing practice (GMP) facility, whilst concurrently entering a long-term partnership with Beacon, to continue manufacturing its products for clinical and commercial use. This secured a dependable and scalable product supply for Beacon, enabling it to focus on the clinical development of its gene therapy pipeline. Beacon is now well positioned as it progresses towards filing its Biologics License Application (BLA) for its late-stage clinical asset, AGTC-501, for the treatment of XLRP.

20,000+
XLRP patients in the US and Europe

1. The UK's MHRA and the EU's EMA have accepted the VISTA study design as being pivotal.

2024 PORTFOLIO HIGHLIGHT

Initiation of registrational trial

Following the positive clinical data published from the SKYLINE trial, Beacon has now initiated its registrational Phase II/III VISTA trial for AGTC-501. The clinical data from this registrational trial will support its BLA in the US and a marketing authorisation application (MAA) in Europe, with the programme now progressing through late-stage development and towards commercialisation.

beacon therapeutics