

Supporting a Rapidly Growing Biotech with a Unique Ocular Gene Therapy

Case Study

An emerging biotech company needed a flexible CRO to support preclinical and clinical development of their viral vector-based gene therapy. Covance helped the biotech Sponsor by performing studies necessary to transition from the preclinical phase to first in human trials and beyond, while adapting to changing needs of a rapidly evolving program. Covance coordinated and streamlined efforts across multiple services including drug metabolism, toxicology, bioanalysis, CMC testing, central labs and early clinical development.

Understanding the Challenge

The Sponsor wanted to work with a CRO that had proven experience in ophthalmology and could help them navigate early development considerations as they were growing exponentially and faced pressures from their investors to quickly advance to the clinic.

The Sponsor initially selected Chiltern as its CRO, which was subsequently acquired by Covance. During this acquisition and seamless transition, the Covance team preserved the program's continuity and maintained best practices for pharmacovigilance and drug safety.

Supporting Preclinical Needs

Supporting the preclinical study design, the Covance Early Development (ED) team learned that the Sponsor had an atypical and difficult requirement to use animal groups both sero-negative and -positive for neutralizing antibodies to the particular AAV-variant administered in the toxicology studies. This presented a challenging study design and procedural matrix- in addition to multiple dosing routes (including subretinal and suprachoroidal) and additional endpoints (including pharmacodynamics & biodistribution), the Covance team developed and used specialized procedures for separate animal housing, handling, dosing, analysis and cleaning to ensure the quality of the results in the study. To support this aspect of the program, Covance relied on its own expert toxicologists and ophthalmologists, as well as those from its partnership with renowned ophthalmologic scientists at [Ocular Services on Demand \(OSOD\)](#). This unusual study design and its high quality results provided insight into strategies for patient recruitment and monitoring requirements for the Sponsor's clinical program.

Key Takeaways

- ▶ Applied specific ophthalmologic expertise to support the viral vector-based gene therapy
- ▶ Delivered high-quality toxicology and bioanalysis results in preclinical development for an atypical and challenging study design
- ▶ Preserved program continuity and seamlessly transitioned the novel gene therapy to first in human trials
- ▶ Established transparency and proactive communications to enable rapid decision making as the biotech's program grew
- ▶ Earned trust to win additional study awards within the clinical program as it enters Phase II/III

Earning Trust

Based on the productive partnership in the preclinical phase, the Sponsor also enlisted the Covance team to support the early dose escalation trials, in which Covance provided regulatory and site services, clinical monitoring, project management, medical services and pharmacovigilance, central labs and bioanalytical support. Covance established clear, actionable study goals and metrics to ensure milestones were met within expected timelines.

Covance instilled confidence in the biotech by ensuring any outstanding issues were resolved and by establishing consistent, proactive communications that enabled full transparency and helped inform the Sponsor's decision making in the rapidly expanding program. Based on this effort and success in the early clinical studies, the Sponsor has chosen Covance to support the next studies in the progression of its clinical program.

Transitioning to a Global Market

Following its acquisition of Chiltern, the biotech was very pleased with the performance of Covance as their full service partner: in addition to the original clinical study, they awarded Covance five additional projects beyond the original clinical studies. As the Sponsor continues to expand, they have asked Covance to also support its vendor management function. The Sponsor also signaled their approval of Covance by asking the team to provide bids on additional studies within the same program as well as for a new gene therapy program targeting a different indication.

The Sponsor is currently focusing on the global expansion of their trials beyond the U.S. as the program moves beyond the early clinical development phase. They continue to view Covance as an extension of their organization and a partner fully aligned with and supportive of its goal to advance these gene therapies to market and make a positive impact on patients' lives.

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