CASE STUDY

End-to-end support to advance gene therapies for rare retinal diseases

As a promising therapeutic candidate targeting rare inherited retinal diseases was spun out of an academic medical center, an emerging biotech needed a knowledgeable and agile partner to provide analytical testing of their product and ensure the project followed an expedited timeline. Learn how Labcorp served as an integral partner in many functional areas as the biologic progressed to the clinic—and how Labcorp later supported clinical operations* for several trials in the program.

Providing biopharm CMC project management as well as GLP, GMP and GCP testing

In the early stages of their business, the emerging biotech first contacted Labcorp to refine their CMC analytical methods, which were initially developed in an academic setting. As a small team, the biotech needed to extend their expertise and relied on Labcorp to provide rapid delivery for non-GMP, GMP, GCP and GLP studies. The biotech also asked Labcorp to provide project management support of their activities, both on the analytical testing side and with their CMO. Labcorp acted as the central analytical laboratory, allowing the biotech to interact with just one partner, thereby streamlining delivery of the results.

As the biotech's success grew, it asked Labcorp to support the CMC testing for four additional candidates in the program, working on each concurrently. Labcorp still had to present a bid defense to earn the contract over competitors; the biotech choose to continue its partnership with Labcorp for each project.

The program started to advance to the clinic as Labcorp prepared the gene therapy for clinical trials with batch release and stability testing to ensure the product was viable for dosing in humans and had a stable shelf life.

During the clinical trial, the Labcorp team supported clinical sample testing, using the methods they had previously developed and validated to test their retinal gene therapy in a variety of sample types such as blood, sweat and teardrops. The trial continued as the team worked quickly to accommodate the biotech's timeline, provide regulatory guidance and quickly support ad hoc requests from regulators for specific tests as the trial advanced.

Supporting a program of complex studies*

From the clinical operations perspective, the biotech initially hired Chiltern, known for its ophthalmology expertise, to support the two programs that reached the clinic. When Labcorp acquired Chiltern in September 2017, Labcorp worked to integrate Chiltern's best practices and standard operating procedures to ensure the continuity of both programs, which had a total of six trials between Phase II-IV.

Supporting non-GMP, GMP, GCP and GLP studies

- qPCR analytical method development and validation
- GLP biodistribution studies (virus and vector) supporting toxicology
- Infectious and genomic titer method development and validation GMP and non-GMP characterization studies
- GMP and non-GMP stability studies
- Potency assay method development and validation
- GCP viral and vector shedding studies—clinical sample testing

*CRO services provided by Labcorp's Clinical Development and Commercialization Services business, which was spun-off into an independent company, Fortrea, on June 30, 2023.

labcorp

Each program contained a feeder trial to monitor the disease progression of the eyes and vision; study participants could then enter one of two interventional trials, where the gene therapy was surgically delivered at one of seven specialized sites in three countries.

With the complex trial network that required 25 referral sites, Labcorp worked to provide oversight at the program level to document best practices and build efficiencies into the program. For example, given the rarity of the indication, many sites were used for multiple studies. Labcorp developed a process for each clinical research associate (CRA) to provide monitoring across multiple studies at one site, rather than having multiple CRAs at one site, enhancing efficiency and continuity. The team also set up program-level meetings with the client to escalate any site-specific issues and share information across the studies to enhance communication and decision making.

While the programs were ongoing, the biotech was purchased by another pharmaceutical company, which already had a long-term partnership with another CRO. Instead of transferring the program of studies to the other CRO, the pharmaceutical company chose to continue working with Labcorp due to its track record of success with the gene therapy program.

Continuing to deliver excellence

Labcorp has proven that it can align a multi-study approach for a complex clinical development program and handle the inherent challenges of an acquisition. The team is due to be awarded a seventh study in the program, providing a full service of solutions, including clinical operations, vendor management, startup, medical monitoring, project management, regulatory, safety, biostatistics and data management. This award affirms the sponsor's decision to keep Labcorp as the partner to efficiently manage these trials despite having a sole-provider relationship with another partner.*

From early-phase analytical method development and validation to first-in-human and late-phase trials, the sponsor has experienced how Labcorp successfully provides end-to-end solutions to advance unique gene therapies, which have the potential to significantly improve the quality of life for people living with rare retinal diseases.

*CRO services provided by Labcorp's Clinical Development and Commercialization Services business, which was spun-off into an independent company, Fortrea, on June 30, 2023.

Learn more at biopharma.labcorp.com/cgt

labcorp