Case Study:

SOLVING OPERATIONAL COMPLEXITY IN EARLY PHASE RETINAL TRIALS

BACKGROUND

Inherited retinal diseases present unique clinical and operational challenges, particularly in gene therapy studies, where genetically defined patient populations and complex safety oversight are required.

The following case study highlights key operational strategies implemented by Medpace to support efficient cohort management, proactive safety monitoring, and strong site engagement. These approaches enabled successful recruitment of a genetically defined early phase cohort of 20+ participants across multiple specialized sites, demonstrating effective enrollment within a highly selective patient population while providing insights applicable to future ophthalmology programs.

CHALLENGES

COHORT MANAGEMENT

STOPPING CRITERIA

SITE ENGAGEMENT

SOLUTIONS

Cohort Management

- Developed timelines and strategies to correctly assign participants to dosing cohorts.
- Encouraged pre-identification of potential participants prior to opening the next dosing cohort.
  - Historical genetic testing and pre-screen genetic testing ICF were used to pre-screen subjects.
- Established a Data and Safety Monitoring Board (DSMB) trio with clinical research experience, surgical management, and biostatistical skill sets.
  - Medpace created a specific charter and data review package for members.
  - Dose decisions were made within 24 hours for dissemination to Sponsor and sites.

Stopping Criteria

- Ensured surveillance of protocol driven stopping criteria and patient safety. When safety events were documented by site staff, they were appropriately flagged to the Medpace team. This immediate escalation allowed for quick action.
- Implemented clear communication pathways to Data and Safety Monitoring Board (DSMB) members, ensuring they were readily available if needed for ad hoc reviews.
- Medpace's strong and nurtured site relationships allowed for open and regular site information sharing that kept sites motivated and recruitment timelines on track.

Site Engagement

- Leveraged well-established relationships with experienced sites that have conducted both retinal surgery and gene therapy clinical trials. Our experience with these sites allowed building on previous relationships to ensure a strong study with quality data.
- Confirmed specific space and staffing for mobility testing and equipment availability to ensure protocol procedures could be completed appropriately.
- Provided support to sites to assist with necessary equipment rentals to ensure compliance with procedures and assessments.

RESULTS

- **Clear structured communication regarding dose cohorts** combined with timely Data and Safety Monitoring Board (DSMB) reviews enabled rapid decision making and minimized delays between cohorts. This coordinated approach allowed sites to proactively identify and prepare eligible participants in advance, supporting timely enrollment as each new cohort opened.
- **Medpace's proactive management of safety events and rapid turnaround of safety reviews** supported timely decision making and minimized disruptions to study conduct. This efficient approach ensured continuity in patient enrollment and helped keep the study aligned with projected timelines.
- **Strong site relationships and consistent communication** ensured the study remained a priority for sites and ultimately drove expedited recruitment within a highly selective patient population.

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a scientifically-driven, global, full-service clinical contract research organization (CRO) providing Phase I-IV clinical development services to the biotechnology, pharmaceutical and medical device industries. Medpace's mission is to accelerate the global development of safe and effective medical therapeutics through its high-science and disciplined operating approach that leverages local regulatory and deep therapeutic expertise across all major areas including oncology, cardiology, metabolic disease, endocrinology, central nervous system and anti-viral and anti-infective.