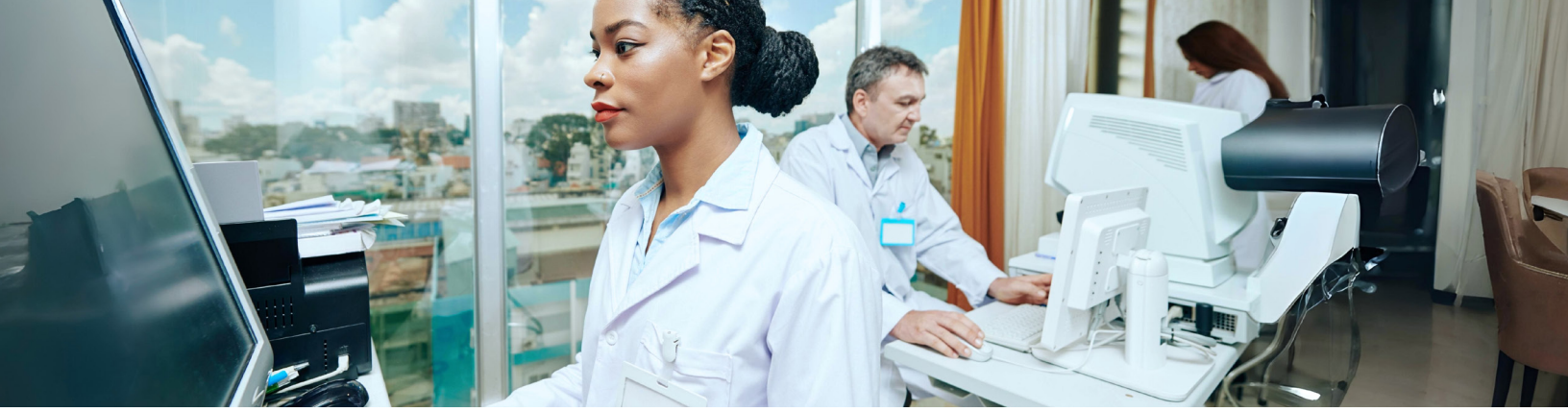
Leveraging Innovative Trial Design to Deliver Geographic Atrophy Study

Advancing Excellence in Ophthalmology Clinical Trials

In a groundbreaking multi-center international study, Emmes was chosen to deliver comprehensive, full-service support for a highly complex clinical trial. This ambitious project aimed to address Geographic Atrophy (GA) resulting from Age-Related Macular Degeneration (AMD), a leading cause of vision loss worldwide.

Spanning 45 months from December 2016 to April 2023, this Phase II prospective, single-arm, nonrandomized controlled trial enrolled patients with GA in one or both eyes. The study's primary objective was to evaluate the difference in the rate of change of the square root GA area on fundus autofluorescence, comparing the 24-month treatment phase to the preceding 9-month run-in phase.

This case study explores how Emmes' end-to-end clinical trial management expertise contributed to the success of this intricate research endeavour, underscoring the company's pivotal role in advancing ophthalmology research.



The Challenge: Navigating Complexities in Trial Activation and Protocol Adaptation

The execution of this Phase II clinical trial faced a significant hurdle prior to activation: a disruption in the manufacturing of the placebo. This unexpected obstacle posed a critical challenge, requiring swift and strategic responses to avoid jeopardizing the study's timeline and objectives.

Adding to the complexity was the need to identify a suitable Investigational Product (IP) manufacturer outside the United States. Securing a reliable and compliant partner for this critical component was essential for advancing the trial globally.

These challenges highlighted the need for :

Emmes Solution: Delivering Agility and Innovation in Clinical Trial Management

Emmes swiftly implemented an innovative trial design to ensure the study could proceed without delays. Leveraging natural history data from each patient's disease progression allowed for a unique approach to analysis, comparing pre-treatment and post-treatment phases. This creative methodology effectively transformed the trial into a single-arm study, overcoming the absence of a placebo.

Leveraging its extensive global network and expertise, Emmes also identified and secured a new Investigational Product (IP) manufacturer outside the United States. The team ensured seamless coordination of the manufacturing process and facilitated timely shipment to the study’s ex-US demographics, overcoming logistical barriers and minimizing impact on the study timeline.

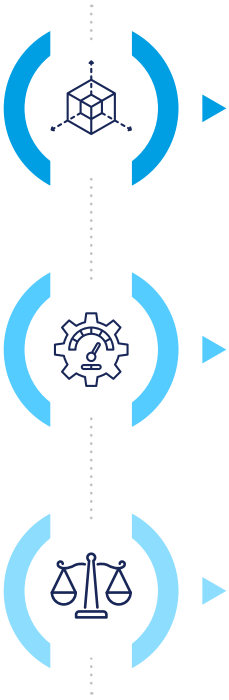
Through these proactive measures, Emmes enabled the successful activation and progression of the trial, underscoring its leadership in delivering comprehensive and resilient clinical trial solutions.

Results and Outcomes

The successful execution of this trial culminated in its publication in the National Library of Medicine, cementing its impact on advancing the understanding of Geographic Atrophy (GA) caused by age-related macular degeneration (AMD). By meeting the protocol objectives established by investigators, the study achieved its primary aim of evaluating disease progression and treatment efficacy despite significant initial challenges.

The innovative study design played a pivotal role in this success, where each patient played dual roles—first serving as their own control during the run-in phase and then transitioning to active participants in the treatment phase. This approach not only preserved the scientific rigor of the study but also provided rich, individualized insights into treatment efficacy.

To address the complexities of this study, Emmes implemented a series of strategic solutions aimed at maximizing data integrity and regulatory efficiency:



Enhanced Statistical Modeling

Emmes designed a robust statistical model tailored to the study's unique challenges. By enrolling 37 subjects, the model effectively leveraged bilateral data, incorporating both pre- and post-treatment measures. Additionally, Emmes utilized historical data as a control, ensuring meaningful insights despite the limited patient population.

Minimizing Bias in Outcome Assessment

To enhance the accuracy of primary outcome measurements, Emmes identified an external Reading Center specifically equipped to grade Fundus Autofluorescence (FAF) images. This minimized potential bias, providing consistent and objective grading across all study sites.

Ethical and Regulatory Guidance

Emmes offered in-depth guidance on ethical and regulatory matters, addressing concerns across the IRB, IEC, FDA, and MHRA. By streamlining these processes and avoiding unnecessary duplication, Emmes facilitated compliance, ensuring the study adhered to global standards while maintaining efficiency and ethical rigor.

Impact

Reflecting on the partnership, our project lead noted,

> "The collaborative execution across our different functional areas and with the Sponsor, sites, and vendors helped conduct the study within timelines. I am looking forward to the dissemination of the study results with the ophthalmic community at large."

The results of this study are now available through publication

For additional information on Ophthalmology Clinical Trials, please visit:
www.emmes.com/ophthalmology