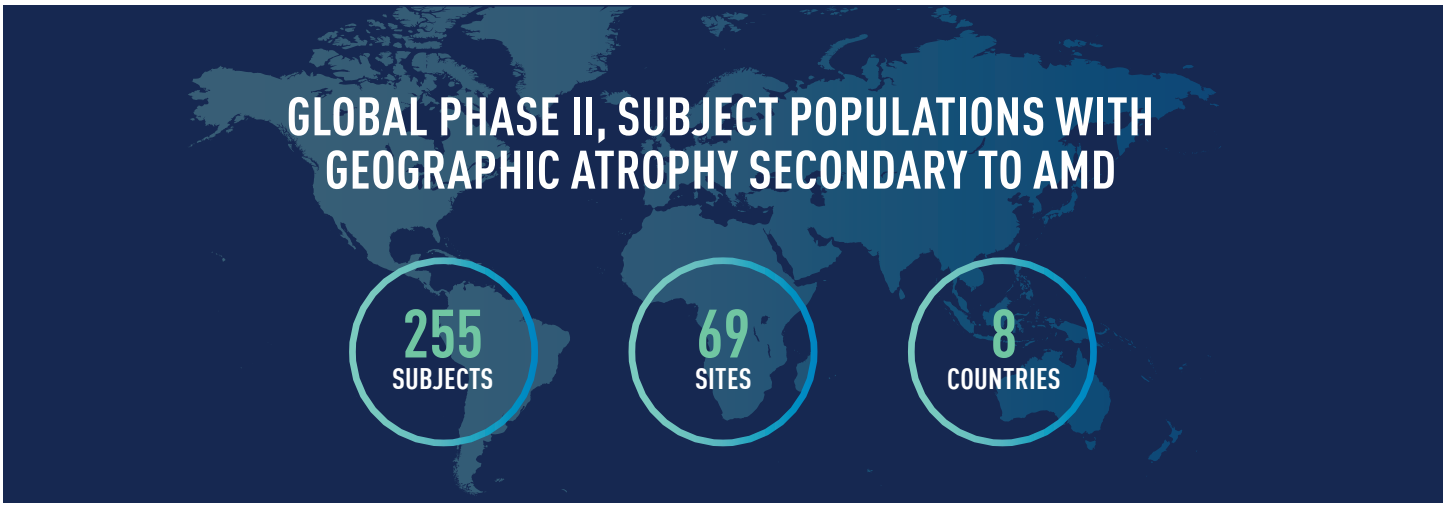
STRATEGIES TO ACCELERATE RECRUITMENT PROJECTIONS IN GLOBAL PHASE II AGE - RELATED MACULAR DEGENERATION TRIALS



SERVICES OFFERED

- Site Activation and Maintenance
- Project Management
- Medical Monitoring
- Clinical Monitoring
- Central Imaging
- Vendor Management

CHALLENGES

The study team faced multiple key challenges during the global Phase II program, including:

GENE THERAPY SUBJECT PERCEPTION AND DOSING PREPARATION

GENETIC TESTING PROCESS AND TIMELINES

IMAGING CENTRAL READER MANAGEMENT AND SITE START-UP TIMELINES

MASKED/UNMASKED MANAGEMENT

RESULTS

Final Recruitment Projections Met 5 Months Early

Medpace's significant ophthalmology and gene therapy experience helped create an efficient and quality-driven program through the proactive mitigation of study associated risks and potential issues. Although COVID-19 impacted site activations and recruitment for the study, start-up timelines and site engagement were key to the success of the program. Several mitigations were implemented, and final recruitment projections were met five months early.

SOLUTIONS

Gene Therapy Subject Perception and Dosing Preparation

- To increase patient enrollment and retention, Medpace developed site training materials and subject education brochures as a tool for patient education on gene therapy to overcome negative perceptions of gene therapy. The patient brochure included information on the indication, how the IP works, potential benefits and side effects. Potential subjects were encouraged by the potential benefits of gene therapy described in the patient brochure, especially the possibility of reversing vision damage or stopping the progression of vision loss with a one-time treatment
- To limit the impact of approval timelines on the overall study timelines, Medpace leveraged extensive experience and strong relationships with sites, Institutional Review Boards, and Institutional Biosafety Committees to ensure the gene therapy investigational product was submitted as early as possible. Medpace provided the sponsor with details on submission requirements to finalize documents early, such as protocol, ICF, IB and the Pharmacy Manual.
- The Medpace team worked with sites early in the selection and start-up process to ensure that preparation of the investigational product was discussed in detail and plans were documented on a site level. Additionally, Medpace worked with sites to ensure that a transport process from pharmacies to the administration site met regulatory requirements. A Chain of Custody log was also provided to sites to ensure proper documentation of transport.

Genetic Testing Process and Timelines

- Working closely with the sponsor to ensure timelines for genetic testing were considered in the protocol and study timelines, Medpace recommended using a genetic pre-screening informed consent when genetic testing took 2-3 months.
- The Medpace team worked with vendors and sites to provide genetic testing kits directly to potential subjects' homes to allow for at-home testing and shipment directly to the testing location.
- Medpace worked with the sponsor to develop genetic testing protocols to be able to filter recruitment into different studies based on genetic results. The genetic testing study included many more sites that would filter potential subjects to sites participating in the study.

Imaging Central Reader Management and Site Start-up Timelines

- To expedite site activation, Medpace Imaging and Operations worked as a cohesive team to ensure efficient training of sites and capture of qualification images. Medpace Imaging worked closely with Data Management to ensure the appropriate capture and transfer of imaging data. Finally, Medpace Imaging ensured the appropriate capture of many imaging endpoints, such as Monochromatic imaging, Optical Coherence Tomography and Microperimetry, while maintaining data accuracy with low protocol deviations.

Masked/Unmasked Management

- To ensure appropriate site resources were in place prior to the start of the study, Medpace communicated early with sites on the requirements for masked and unmasked study staff. Additionally, Medpace worked with the site to ensure an appropriate plan for dosing and efficacy assessments were developed and documented in the site-specific Masking Plan.