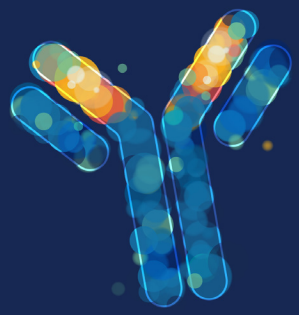
Case Study:

OPTIMIZING STARTUP AND ENROLLMENT FOR A SUCCESSFUL GLOBAL PHASE III HEREDITARY ANGIOEDEMA (HAE) STUDY



51 SITES

91 PATIENTS

14 COUNTRIES

(Belgium, Bulgaria, Canada, Denmark, France, Germany, Israel, Italy, Netherlands, Poland, Spain, Turkey, UK, US)

CHALLENGES

- Clinical Study Agreement (CSA) and budget delays
  - Delayed institutional reviews and budget negotiations
  - Extensive institutional signature timelines & wet-ink requirements
  - Reviews gated by site negotiation fees
- Regulatory delays
  - Queries
  - ICF revisions
- Delayed activation shortened screening window
- Delayed recruitment activity following activation
- Reduced recruitment rates
- Pediatric/adolescent challenges

THE SOLUTIONS

Implemented a range of mitigations during startup and enrollment to spur engagement, minimize patient and site burden, and ensure study milestones remained on track.

- **Startup Mitigations:** Introduced tactics to speed up CSA/budget negotiations
  - Moved to electronic CSA signatures where permissible
  - Worked with PIs to increase institutional pressure to review documents
  - Developed competitive budget to ensure the study would be attractive to investigators, and support faster negotiations
  - Leveraged institutional intelligence and lessons learned to draft text that was less likely to need revision
  - Offered ad-hoc teleconference support calls to sites
- **Proactive Regulatory Mitigations:**
  - Maintained Q&A document to prepare for anticipated/reoccurring queries
  - Mapped submission/approvals/query timelines so the team was aware of deadlines and could anticipate workload
  - Held ad-hoc team meetings to triage responses
  - Held Medpace/Sponsor onsite visits to increase engagement and visibility
  - Leveraged Investigator Meeting (IM) attendance to break roadblocks in negotiations
  - Defined backups and escalation pathways
- **Recruitment Mitigations:** Implemented a tailored site engagement approach
  - Rescue medication reimbursement
  - Hosted multiple IMs (in person and virtual) to add flexibility for sites
  - Launched campaigns to receive referrals from Sponsor call center, advocacy groups, other third-party vendors
  - Hosted site engagement calls, including a PI Forum
  - Developed site newsletters with enrollment recognition to spur competition
- **Provided Services to Support Patients and Lessen the Burden**
  - Included vendors to provide:
    - Direct to Patient IP shipment
    - Patient concierge and travel reimbursement
    - Home Health visits
    - Patient call center
  - Provided robust patient stipends for time/effort
  - Developed a menu of patient/site items, including a study website
- **Other Recruitment Initiatives**
  - Provided updates on publications from Sponsor
  - Offered assistance with chart review and staffing solutions
  - Conducted Medpace/Sponsor booster visits
  - Utilized the ClinTrak® Prescreening portal to track potential subjects
  - Developed Site-Specific Recruitment Plans with targeted follow up and goals
  - Developed media/digital recruitment campaign options

RESULTS

Site engagement increased and new sites/Investigators contacted the Sponsor requesting to take part in study

While startup was initially delayed, all sites were activated ahead of the projected date

Ended recruitment ahead of schedule with sites requesting that the Sponsor add additional enrollment slots for their prospective patients

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.