Case Study:

ADVANCING THE TRANSLATIONAL DEVELOPMENT OF A NOVEL BIOLOGIC INTO A FIRST-IN-HUMAN CLINICAL TRIAL FOR A RARE DISEASE

BACKGROUND

A rare disease is classified by the US Food and Drug Administration (FDA) to impact fewer than 200,000 patients nationally; representing a very small fraction of the population. Collectively, hundreds of millions of people are diagnosed with rare diseases globally, spanning more than 7,000 individually recognized clinical conditions. Early phase clinical trials for these diseases can involve complex designs to assess the safety of the investigational drug, its maximum tolerated dose (MTD), pharmacokinetics (PK), and pharmacodynamics (PD) after administration of single and multiple doses.

Medpace partnered with a biotech company to execute a sophisticated Single Ascending Dose (SAD) and Multiple Ascending Dose (MAD) clinical trial requiring a large healthy volunteer population enrolled exclusively at its Phase 1 Clinical Pharmacology Unit (MCPU).

STUDY OVERVIEW

- Randomized, placebo-controlled, blinded, dose-escalation Phase I study
- Single Ascending Dose (SAD) and Multiple Ascending Dose (MAD) design
- Large healthy volunteer population with multiple cohorts enrolled at the Medpace Phase I Unit

RESULTS

Enrollment target achievement:	Enrollment Rate:	Time to first participant enrolled:	Visit Compliance Rate:	Retention Rate:
100%	~13 participants / month	5 days	98.4%	96%

- **Enrollment Achieved on Schedule:** Successfully enrolled a large healthy volunteer population into a complex, long-duration first-in-human study – spanning 7-9 months per subject – while maintaining planned study timelines.
- **High Protocol Compliance:** Despite an intensive study design requiring numerous inpatient and outpatient visits, the study achieved excellent protocol and visit compliance, supporting high-quality clinical data.
- **Successful Execution of Complex Assessments:** Coordinated specialized imaging procedures alongside traditional Phase I assessments, enabling collection of critical endpoints without disrupting study conduct.
- **Strong Sponsor Partnership:** Through proactive communication, operational flexibility, and rapid problem-solving, the study team consistently delivered solutions that maintained study momentum and received positive Sponsor feedback throughout execution.

CHALLENGES

- **Complex First-in-Human Study Design:** The study required a relatively large healthy volunteer population, rigorous inclusion and exclusion criteria, and lengthy participant commitments across multiple inpatient stays and outpatient follow-up visits.
- **Specialized Imaging Requirement:** Study endpoints required MRI and DXA imaging at qualified external research facilities capable of performing protocol-specific assessments, creating additional startup, scheduling, and logistical complexity.
- **Participant Recruitment and Retention:** Recruiting healthy volunteers willing to participate in a long-duration study with numerous study visits and specialized procedures required targeted engagement and retention strategies.

SOLUTIONS

STRATEGIC RECRUITMENT AND RETENTION

- Leveraging an extensive healthy volunteer database and experienced Phase I recruitment team, Medpace implemented targeted recruitment initiatives, dedicated participant communication, and proactive follow-up to support enrollment and retention throughout the study.

COLLABORATIVE OPERATIONAL PLANNING

- Medpace partnered closely with the Sponsor to optimize eligibility criteria and visit scheduling where appropriate, improving participant experience while maintaining study integrity and subject safety.

INNOVATIVE VENDOR COORDINATION

- The study team rapidly identified, evaluated, and qualified specialized providers capable of meeting protocol-specific imaging requirements. Through proactive coordination and flexible scheduling, Medpace successfully integrated these imaging procedures into the study workflow while minimizing operational risk and avoiding protocol deviations.

PROACTIVE, SOLUTION-ORIENTED STUDY MANAGEMENT

- When operational challenges arose, the project team consistently identified practical solutions – including adjusting assessment windows and coordinating outpatient visits – to keep the study on track. This collaborative, flexible, and “out-of-the-box” approach was recognized by the Sponsor as a key contributor to the study’s success.