Over-Enrolling a Challenging Rare Disease Study:

Emmes Left No Patient Behind

Enrolling patients into a global rare disease study is challenging under any circumstances. But for one Sponsor with a pivotal study in neurology, the hurdles were daunting. The situation called for the kind of foresight and comprehensive recruitment approach that Emmes's rare disease team could bring.

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Case Study

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The Challenge: Unrealistic Enrollment Projections and a Limited Patient Population

Because the study was in a competitive therapy area and the disease was rare, the pool of patients and qualified study sites was limited from the outset. Additionally, several extraordinary circumstances combined to make study participation a “hard sell” for patients:

- The protocol itself involved several clinical visits, many of them lasting all day
- The visits entailed multiple procedures that required coordination across departments, and they, therefore, had to be booked months in advance.
- Some patients had achieved a stable disease state and so were unwilling to try an experimental therapy.
- The study was enrolling during the early days of the pandemic when patients were reluctant to visit clinics (and indeed, many were closed in several countries).
- Sites, especially those in the US, have historically been over optimistic about their recruitment potential and the Sponsor was basing its planning on those projections.
- The inclusion/exclusion criteria within the protocol suggested that there would be a high screen-failure rate. The expectation at the start of the trial was that about 25 percent of patients would fail the screening.

The Emmes Solution: Leave No Patient Behind

The Emmes feasibility team recognized at once that, based on their experience, the initial patient numbers that sites were anticipating was unrealistic. Due to staffing shortages, sites in all countries had a limited ability to recruit patients. Emmes worked with a vendor to supplement sites' resources, in line with the contracting process.

To minimize the risk that the trial could not recruit according to the Sponsor’s schedule, Emmes recommended and activated sites in two backup countries, which ended up contributing **15% of the overall recruitment numbers.**

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The Emmes study team then adopted a multi-prong strategy to ensure that every eligible patient was given multiple opportunities to enroll and that sites remained engaged with the study. The approach included:

- Communicating regularly with sites to keep the study and their enrollment efforts top of mind. The Emmes project manager was in frequent, direct contact with investigators, as were the Clinical Research Associates (CRAs), and often the Sponsor. This was especially important at big institutional sites where the normal channels of communication are with centralized departments with competing priorities.
- Engaging recruitment organization KnowRare, alongside Patient Advocacy Group involvement and KoL engagement with the sponsor to ensure as many patients as possible were made aware of the study.
- Revisiting pre-screening logs and having sites re-contact patients who were initially unable to participate for logistical reasons to see if their situation had changed in the interim.
- Offering a concierge service to minimize patients' inconvenience in visiting sites.
- Meeting weekly within Emmes, and monthly with the Sponsor to brainstorm solutions to site-specific challenges as they arose.
- Involving the Sponsor. The Sponsor appointed a dedicated site engagement team member, and the Sponsor's medical director visited sites as needed to maintain awareness of the study.

The Result: Exceeded Enrollment Target, Contained Costs

While the stated goal was to enroll **200 patients**, Emmes actually over enrolled the study with **210 patients**, providing a cushion to counteract any patient drop out. The enrolled patients were distributed uniformly across **43 sites in 15 countries**. This performance above-target was remarkable given the circumstances – most notably that the screen failure rate was dramatically higher than anticipated; rather than the 25 percent expected, in reality it was between 35 percent and 40 percent. By analyzing the trends and discussing adjustments to the study design and eligibility criteria with the Sponsor and the medical team, the Emmes team was able to successfully prepare for – and overcome – even such unforeseen obstacles.

In respecting the value of every potential participant, Emmes managed to complete enrollment according to a revised schedule, for which the Sponsor was grateful. The comprehensive recruitment strategy kept the Sponsor's costs from escalating due to significant recruitment delays. And, in being able to accurately forecast the trial end date, the emerging company was able to avoid a public setback that could have affected its stock price.

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