PRA Health Sciences

CASE STUDY

Creating Solutions to Ensure Endpoint Protection in Challenging Rare Disease Trial

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STUDY DESCRIPTION

A sponsor engaged with PRA to implement the first clinical trial in a specific rare disease.

Primary Endpoint:
Safety, tolerability, and efficacy of medical product

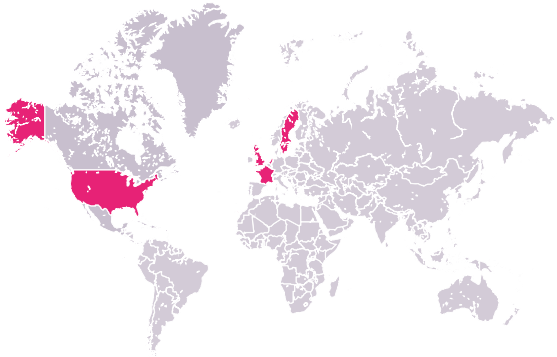
Study Phase:
Phase II

Study Duration
44 months

No. of Clinical Sites
18 sites

Patient Population
24 patients

Regions
France, Netherlands, Sweden, United Kingdom, United States



Situation

The collection of high-quality endpoint data was paramount to the success of this Phase II program. Because this was the first clinical trial ever done for this disease, it was necessary to develop non-disease-specific clinical assessments, in collaboration with Key Opinion Leaders (KOLs), for use as endpoints. The newness of these tools presented challenges to site selection and training, as well as risks to uniformity in patient recruitment and consistency in data collection.

Challenges

Seven leading KOLs were selected by the sponsor to participate in this rare disease trial. Physician-to-physician communications generated enthusiasm from potential research sites to be involved in the clinical trial and resulted in requests for the study to come to additional sites and countries. This organic growth was well received by the sponsor, and the study expanded its global reach beyond original projections of 7 sites in the US to include 18 sites in 4 countries.

The challenge was to emphasize on all participating and interested investigators the need to minimize variability, and to develop methods to ensure standardization in all data collected for clinical endpoints.

Solutions

To ensure that the data from every rare disease patient would be evaluable and high-quality, the sponsor required a process that minimized noise in the data and ensured standardization in all patient assessments. Team Members from PRA's Medical Affairs and Clinical Operations partnered with the sponsor to develop a clinical evaluator manual outlining each step to ensure standardization in assessment and data colleciton.

Team members from PRA's Medical Affairs and Center for Rare Diseases partnered with the sponsor to create a detailed clinical evaluator qualification and certification process, including a training and certification program, that took into account varied expertise and experience levels required for each clinical endpoint assessment.

PRA worked with the sponsor to:

- Devise a methodology of minimum qualifications (education, research experience, and therapeutic area experience) for a clinical evaluator, with qualification requirements adjusted as appropriate for each study endpoint
- Establish a requirement for the number of clinical evaluators at each site
- Provide in-person training
- Develop an online training program for site personnel who could not attend the in-person training
- Develop and implement a proficiency-testing program to ensure only adequately-trained clinical evaluators were certified to collect data for each endpoint
- Establish rules to demonstrate continued proficiency throughout the study period

Team members from PRA's Medical Affairs and Clinical Operations developed a data review process to quickly identify data variances and developed mitigation strategies with the Center for Rare Diseases team if site re-training was needed.

Results

Despite the growth of the study from the originally planned 7 sites in the US to 18 total sites in 4 countries, the clinical evaluator qualification and certification process was rolled out smoothly and ensured standardized quality training was provided to all sites. The collaboration between team members from Medical Affairs, Clinical Operations, and Center for Rare Diseases enabled PRA to provide the sponsor with a solution that is unique in the rare disease space and ensure that the endpoint data obtained are of highest quality.