



CASE STUDY

PRAHEALTHSCIENCES

PRA'S FEASIBILITY LEADS TO UNIQUE ENROLLMENT STRATEGY





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PRA recently performed a feasibility study to identify investigators who see patients with a rare neuromuscular autoimmune disease and who have the capability to perform a Phase II clinical trial. It was estimated that there may only be 1,000 to 3,000 patients in the US and Europe with the condition.

The major challenge that PRA faced was identifying the investigators and sites that see these patients. The protocol required 42 patients, and it was initially estimated that at least 35 sites would be needed to enroll these patients. The feasibility team selected 18 countries in North America, Latin America, Western Europe, Central and Eastern Europe, and Asia. Country selection was based on data from PRA's investigator database, input from our local country experts, and Medical Informatics data searches that showed where the greatest numbers of physicians treat or study patients with the disease.

PRA was able to identify a total of 88 interested investigators during the feasibility study. The feasibility questionnaire provided information on patient volumes and standard of care, input on the initial study design, and feedback from investigators. These data were critical to the sponsor's ability to plan the study since PRA was able to show how many patients were available and where the greatest numbers were located. Based on the feasibility data, PRA's medical and operational teams worked with the sponsor to develop a unique, data-based strategy for the clinical program.

A key message from this experience is that rare diseases patient populations and orphan drug indications may be one of the most pertinent areas in which to perform a feasibility study. We were able to "cast a wide net" (18 countries and over 600 sites) to identify over 80 sites where the target population was treated. Given the paucity of data on this disease in the public domain, especially concerning patient numbers, the information gained during the feasibility study—directly from physicians who treat these patients—provided the sponsor with a unique source of treatment data.

Another key observation was that investigators who treat rare diseases may be more motivated to participate in clinical trials given that the indications are not ordinarily a major focus of drug makers (based on their smaller market potential).

"In order to engage rare diseases patients and their caregivers, it is critical to first understand what it's like to walk in their shoes."

When working with patients on rare disease clinical trials, it is not only important to focus on finding these patients, but reaching them by understanding their needs and supporting their journey."

**– GRETCHEN GOLLER, MSW SENIOR DIRECTOR
PATIENT ACCESS AND RETENTION SERVICES, PRA**

PRA Health Sciences conducts comprehensive Phase I-IV biopharmaceutical drug development.

To learn more about our solutions, please visit us at prahealthsciences.com
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