

RARE DISEASE

Familiar challenges. More challenging solutions. And we can deliver them. It's what we do. Best.

It's all about having the right experience, and plenty of it. With 115 rare disease studies conducted in the past five years, across more than 50 indications and in multiple countries and languages, Premier Research has become the clinical development services company of choice for sponsors who know they can rely on us to complete incredibly complex rare disease studies successfully.



RARE DISEASE

Overcoming the challenges of rare disease trials

It's more a matter of broad experience than of learning a set of rules. We can help you understand what you really need to think about to complete your study successfully.

- + The number one challenge: identifying, recruiting and retaining patients from the miniscule number affected by the disease
- + Equally critical: the challenge of selecting effective sites and motivating investigators
- + The regulatory challenge of applying for and achieving Orphan Drug status
- + The challenge of treating a small number of patients who may be spread across a large number of countries
- + The challenge of maintaining effective communications
- + The challenge of keeping the study on track despite endless obstacles
- + The challenge of pulling it all together and delivering accurate results

Overcoming a unique degree of difficulty in patient recruitment

Patient recruitment is the number one challenge for virtually all clinical trials. But it's a completely different challenge when there may be only 50 or 100 qualified patients in the whole world.

For some diseases, where treatments are of relatively short duration and relatively few follow-up visits are required, we may fly patients and their families from, say, the Middle East to a treatment center in the UK. The logistics are complicated, and regulations may require us to obtain approvals from ethics committees in both countries. But when the situation calls for it, we have the experience to get it done.

We have conducted **115** rare disease studies in the past five years, across more than **50** indications.

Drug development for rare diseases faces many of the same challenges as other drug development projects. But rare disease studies are not miniature versions of standard trials. They face unique obstacles, and we have the kind of hands-on experience it takes to both anticipate and overcome the obstacles. Some people may call rare disease studies a niche. Our project managers call them their life's work. It's second nature to them to pull together the right resources, from therapeutic and regulatory expertise within Premier Research to advocacy groups and KOLs from the necessary countries. They understand the big picture, and keep their eyes on the details. It's the kind of specialized experience you can expect at Premier Research. It's what we do. Best.



IT'S WHAT WE DO. BEST.™