Published in February 2018
PharmaVOICE


# Rare Disease Research Operational Risk: Supporting New Clinical Sites

Over the last decade, the number of drugs gaining FDA approval for new indications per year has nearly quadrupled, growing from seven new or expanded approvals in 2007 up to 21 in 2017. As more rare diseases are identified and rare disease research expands to new indications, it must also expand to reach remote clinical sites that have little to no clinical research experience.

This presents several operational challenges, but creating and investing in solutions can be seen as an opportunity for the future. To start, sites without research experience can be more difficult to identify and qualify. Once identified, significant challenges include estimating the level of interest/engagement by the Principal Investigator (PI), implementing physician and non-physician training, and building a research infrastructure at the site, including Good Clinical Practice (GCP) training, protocol training, and recruitment strategies and tactics. While it can add time to activate a research-naïve site, the investment positively impacts enrollment in the overall timeline. Finally, it's critical to have the resources and capacity necessary to adequately support a clinical site that is new to research. Anything less than a positive experience can turn a new investigator away from participating in a clinical trial again in the future. More importantly, ongoing support tailored for sites new to research is crucial for patient safety, enrollment, and retention.

## Identifying and Qualifying Research-Naïve Sites

As challenging as it is to find qualified rare disease research patients, it can be just as challenging — or not feasible at all — to locate research sites with experience in trials specific to rare indications. In these instances, it's common to rely on executing a protocol with a majority of clinics that are naïve to conducting clinical research. Qualifying sites without clinical research experience, or with reluctance to participate, requires additional considerations and processes outside of standard site qualification SOPs. Don't forget to consider these questions especially when qualifying research-naïve sites:

**What infrastructure exists to successfully execute the protocol and what's missing?**
Beyond equipment, storage space, lab access, etc., it is also important to know what referral infrastructure and connections exist with hospitals, medical practices, and advocacy groups that can be leveraged to maximize trial awareness. Also, be aware of what needs patients have specific to the type of indication that are beyond standard requirements, travel and logistical support, and if the site is prepared to handle those needs.

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**Who specifically will serve as the site's primary research coordinator?**
It is important not to assume that there is staff available to centrally manage the activities of a coordinator, and if there is not a resource, create a plan to subsidize one.

## Preparing Sites for Research

On top of ensuring sites are prepared with the required physical infrastructure, site staff must be well informed on regulations within the Code of Federal Regulations (CFR) Title 21, GCP principles and processes, as well as the basics of clinical research, like expectations for study monitoring visits and working with IRBs or IECs. While there are free training resources available, such as the National Institutes of Health (NIH) GCP training certification, it's important to be prepared to provide GCP training and have methods in place to ensure that training is documented.

## Supporting Clinical Sites New to Rare Disease Research

The study team must be prepared to provide continuous support beyond the start-up phase. This is particularly important in rare disease studies because a higher percentage of sites may never enroll one patient due to scarcity, and this can impact site engagement.

Maintaining a positive experience for sites in this type of circumstance requires transparent communication, ongoing encouragement and a consultative, site-by-site approach for supporting patient recruitment efforts.

As research of investigative products for new rare disease indications accelerates, there is a growing necessity to work with research-naive clinical sites. While this presents inherent operational challenges, there is a greater opportunity at hand. In broadening networks to include more research-naïve sites, the industry is responding by improving clinical research education for patients and medical practitioners, refining tools and technology for clinical site training, and strengthening strategies for patient recruitment and retention in rare disease studies by leveraging new tactics.

With more new clinical sites' willingness to learn and adopt clinical research infrastructure, more therapies and treatment options will be made available for rare disease patients who need it most. PV

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