

Clinical trials for rare diseases: Challenges and Solutions

Many rare diseases are lethal, quickly progressing and debilitating. For this reason the principles of Good Clinical Practice (GCP) and Food and Drug Administration (FDA) regulations ask for **adequate and on-time responses which will prevent as many fatal consequences as possible**. However, the reality works quite the opposite way. One of the biggest difficulties is the delivery of positive results in a short period of time since clinical trials for rare diseases can take years. This puts at risk a huge number of people all around the world. Because the nature of such conditions is harder to identify than the nature of non-rare diseases, researching the formal ones becomes **a process which advocates a struggle in terms of finding the balance between benefits vs. risks, pace vs. safety as well as time vs. results**.



The whole process can be speeded up when more people take part in studies which are focused on rare diseases. Nevertheless, here arises another deep problem as **participation in such researches is rated to be far less than participation in other researching and testing procedures**.

There is a certain problematical tendency which has been circulating across time. Back in 1962, the tempo of new pharmaceutical product development was perceived to be too rapid and hurried. This was in regard to the thalidomide tragedy. Produced as a sedative which was said to be efficient in terms of relieving morning sickness symptoms, the thalidomide turned out to have catastrophic side effects. The consumption of the medicine by pregnant women caused birth defects. Consequently, the Kefauver Harris Amendment asked for **more substantial testing, informed consent of patients and statistical proofs**.



Later on, around 1980's drug development procedures were progressing way too slow while the AIDS epidemic was striking very quickly and was taking more and more victims. In response to the global alarm caused by the disease and the lack of existing treatment, the Orphan Drug Act required **expediting drug development to fight the disease**. About twenty years later in 2002, the White House signed the [Rare Disease Act](#) that prioritized rare disease research.

Taking part in clinical studies that are centered on **dealing with rare diseases is essential but recruitment of participants has transformed into one of the biggest challenges in the Pharmaceutical and Medical industry**. With not enough suitable patients, the task becomes just as difficult as producing a perfectly efficient drug for the shortest period of time. In recent surveys, 2,759 trials out of 24,088 were categorised as rare disease trials which were more likely to enroll under 50 volunteers. What is more, according to an analysis of [ClinicalTrials.gov](#), such studies were most often non-randomised, single arm and open label.

In their attempts to make it easier for more people to enroll, sponsors and trial managers **seek alternative approaches and strategies**. In the prevailing number of different cases, patients point out the inconvenience of traveling as the main reason for their lack of interest or refusal to participate. To tackle this, various organizations make it possible for in-house clinical services. This way, many clinical processes like blood draws, checks upon the health conditions of patients and treatment administration, can be carried out by a clinical nurse who travels to the specific location for the ongoing trial. In-home supported clinical studies, then, **help reduce two of the biggest hindrances and obstacles for conducting clinical projects with more participants: time and cost**. By offering comfort to patients in terms of those two key aspects, researching rare diseases and finding potential

solutions in the form of drug products become more productive and easy to complete activities for researchers.



Another way to ease the process of finding suitable candidates and recruit them is by looking for patient support groups. Since they keep patient registers, recruiters and research experts are enabled to find information about the location of specific subjects, their health conditions, biological sample data and genetics. Furthermore, **contacting such groups can also help sites to cover bigger geographical areas** and to discover other treatment centers and research specialists who could provide with further assistance in a relative clinical study.

To sum up, clinical trials **are vital for the improvement of people's welfare and for the overall progression of the Pharmaceutical, Clinical and Medical field**. Nevertheless, fewer people are stated to want to participate in clinical studies for rare diseases. The reason behind this is the inability of individuals to cover all the expenses for the travel to the location where the trial is going to be implemented. The other factor that prevents them from **taking part in a study is the fact that it is too time-consuming**. To deal with these setbacks, researchers and sponsors seek for different and more convenient solutions like organizing in-house trials and contacting patient support groups.

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