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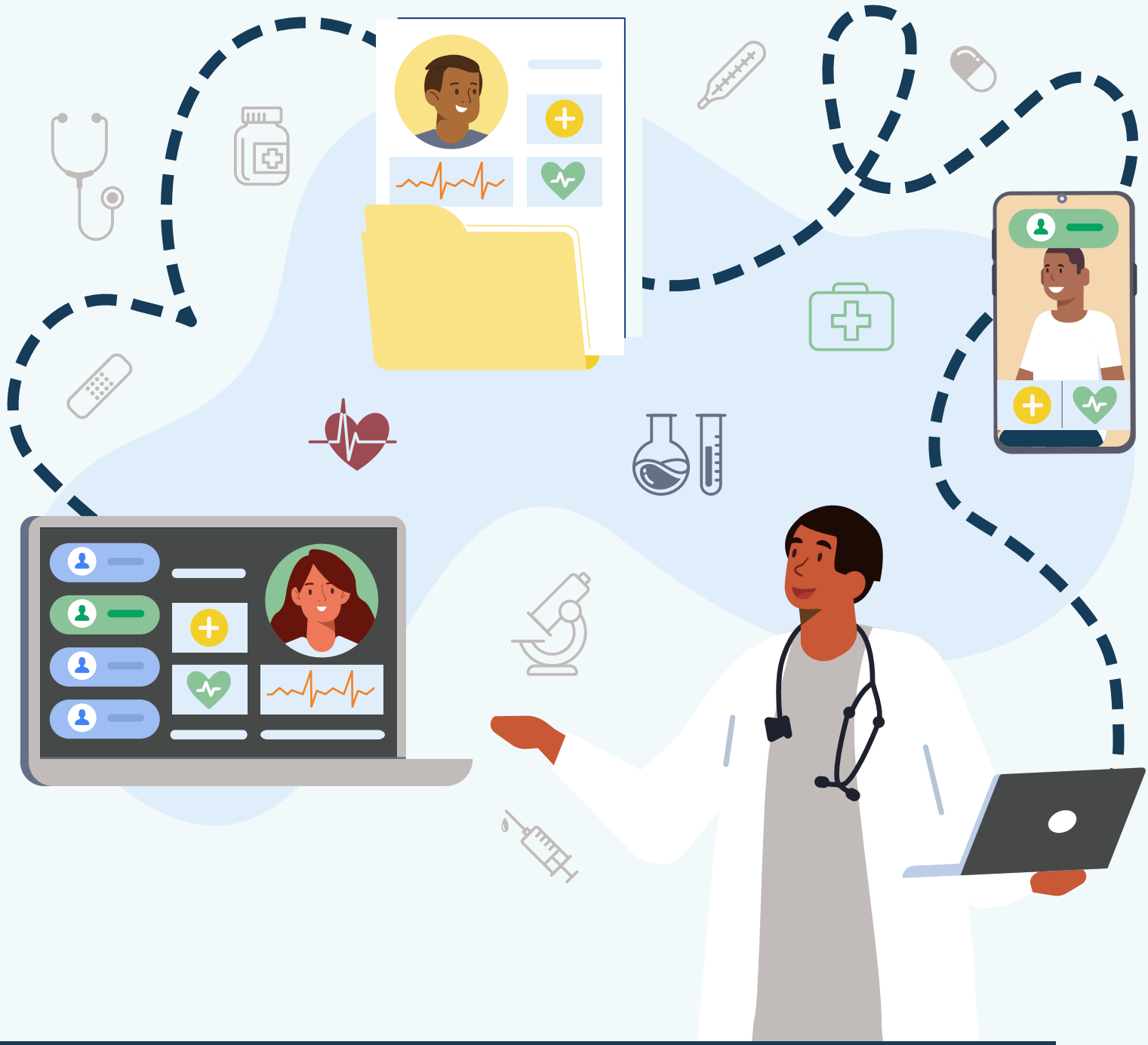

Understanding the Art and Science of

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Industry Standard Research

UNDERSTANDING THE ART AND SCIENCE OF PATIENT RECRUITMENT

INTRODUCTION

Are Your Patient Recruitment And Retention Strategies Putting The Success Of Your Trial At Risk?

ED BILLER

Patient access and recruitment are ongoing bottlenecks in today's clinical trials, with many sponsors experiencing delays, and even failure, as a result of poor enrollment and/or high dropout rates. Now, as clinical trials become increasingly complex, it is more important than ever to understand how to overcome these challenges in order to accelerate timelines, lower costs, and improve the quality of clinical trial data.

Recognizing the need for more insight on how to navigate this evolving landscape, Industry Standard Research (ISR) has been collecting data on a wide range of areas related to patient access and recruitment. This includes but is not limited to the challenges of finding and retaining clinical trial participants; what specific attributes clinical development outsourcers are focusing on when it comes to securing a reliable provider; and strategies that can help diversify patient recruitment approaches.

This e-book examines the results of this effort, focusing on the use of specialized recruitment companies as one possible strategy and also discussing the nuances associated with rare disease studies and their key differences when compared to more traditional studies. For example, smaller patient populations, among other challenges, mean sponsors must cast a wider net for patient recruitment as well as integrate flexibility for retention. And with an estimated 7,000 rare diseases known to exist and more than 90% without an FDA-approved treatment available[1,2], this area offers many opportunities to advance healthcare and enhance the quality of life. However, doing so requires sponsors to find enough patients to help expand the industry's knowledge about the efficacy of these therapies. Therefore, our hope at ISR is that this information provides sponsors with a valuable guide for effectively finding and retaining the patients needed to explore today's innovative pipeline, as we all work together toward improving and accelerating the future of drug development. ISR

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1. National Organization for Rare Disorders (NORD). May 11, 2002. NORD Applauds Legislative Efforts to Restore Intent of the Orphan Drug Act. *https://www.prnewswire.com/news-releases/nord-applauds-legislative-efforts-to-restore-intent-of-the-orphan-drug-act-301545602.html*

2. FDA. (May 16, 2022). Rare Diseases Cures Accelerator. *https://www.fda.gov/drugs/regulatory-science-research-and-education/rare-disease-cures-accelerator*

Can CROs Rise To The Patient Access & Recruitment Challenge?

SHERRY HUBBARD-BEDNASZ Market Research Director, Industry Standard Research

You have a human condition to improve and a protocol to follow. All smooth sailing from here, right? Indeed, even the most well-planned clinical trial is dead in the water if the needed patients are not accessible or willing to participate.

Yes, we've been saying it for some time, that the business of conducting clinical trials is becoming more complex. Believe me, in our market research world, respondent fatigue, nonresponse, and sampling snags are real issues that affect our research goals. These also apply to clinical development, granted in a far more intricate manner. Need humans? Welcome to the challenges of dealing with them.

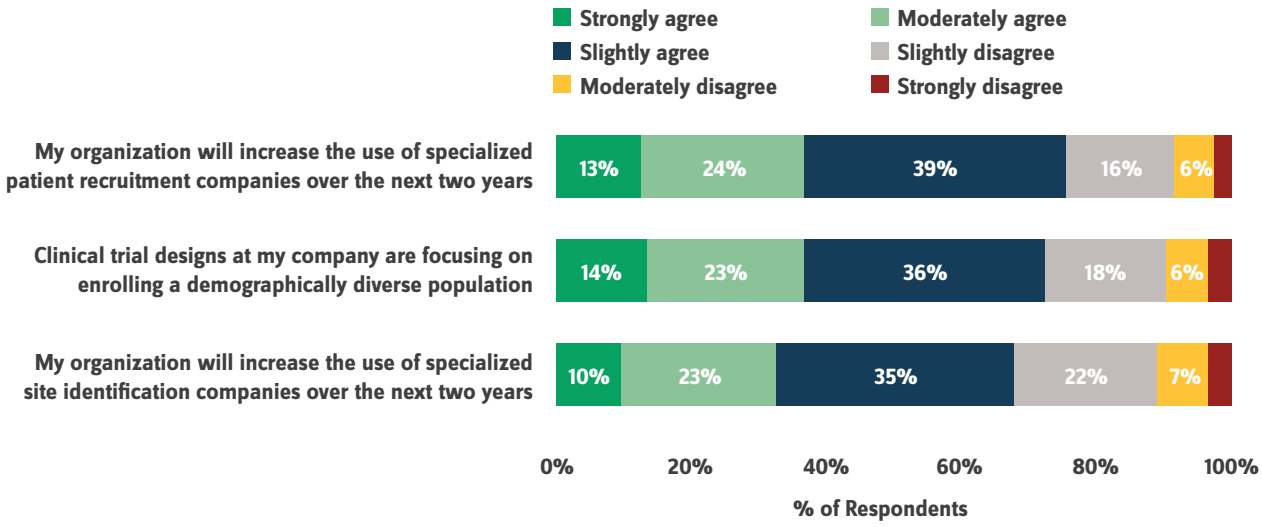
Let's add another layer: outsourcing. Such a small word to depict sizable decision-making. When it comes to clinical trials, it takes a village. And a lot of that village is outsourced. Whether you're a veteran or new to this industry, we at ISR aim to take some of the mystery out of companies' outsourcing decisions. Where do you start? What do you need? In this vast landscape of service providers, is there a match for you? Every year we survey hundreds of clinical development outsourcers to share their perspectives and experiences regarding two of the most challenging and time-consuming processes: CRO selection and performance evaluation.

It is not surprising that the task of CRO selection is often stress-inducing for many companies determining which provider is best-suited to conduct their trials. The desire to find the neon flashing sign pointing to all the answers is understandable. Unfortunately, that sign doesn't exist. Many factors are at play when it comes to provider selection. One element we explore in depth per our annual CRO benchmarking survey is the influence of preferred provider agreements or the lack thereof. We ask clinical development outsourcers about the attributes they focus on under three selection environments: 1) using preferred provider agreements, 2) going outside of preferred provider agreements, and 3) no preferred provider agreements.

While these selection environments are different, all three decision-making scenarios tend to share some common pain points year over year. One commonality that stood out this year: patients. For Phase 1 outsourcers, and for the third year in a row, access to patient populations was a universally shared attribute across the three scenarios. For Phase 2/3 outsourcers, patient recruitment strategy was a universally shared selection driver — and a newcomer on the shared scene. When asked to give a reason for their provider satisfaction ratings, several respondents called out a good or bad experience on the specific aspect of patient recruitment. This is proof of the importance and expectation that companies have when it comes to tracking down patients.

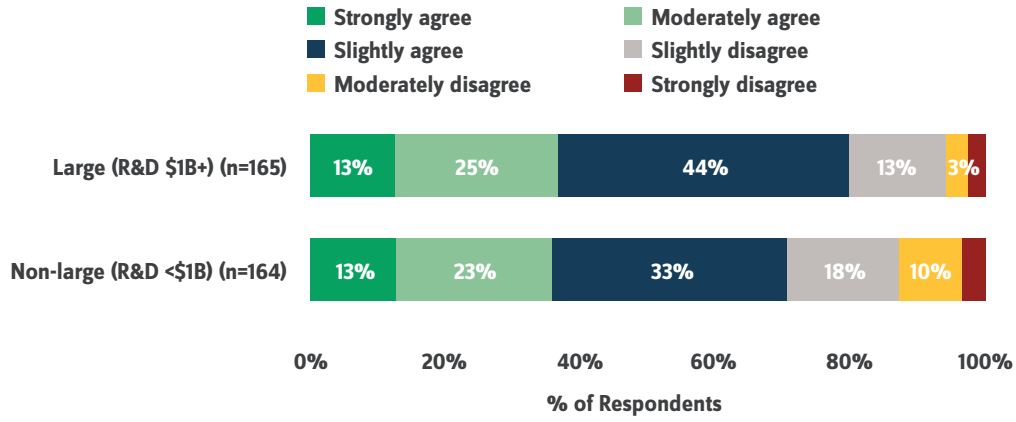
	Phase I Outsourcers *Access to patient populations* selected as a Top 5 attribute	**Phase II/III Outsourcers** *Patient recruitment strategy* selected as a Top 5 attribute
Using PPAs	**29%** of respondents	**31%** of respondents
Going outside of PPAs	**31%** of respondents	**30%** of respondents
No PPA agreements	**29%** of respondents	**22%** of respondents

We take the patient-related selection driver a step further with a look at companies' future plans. We asked more than 300 outsourcers their level of agreement with statements related to their clinical development programs. Two-thirds to three-quarters of respondents indicated future expectations for using more specialized patient recruitment and site identification companies and enrolling demographically diverse populations. Moreover, half of these respondents moderately to strongly agreed with these expectations. Important to note, these sentiments were collected in November and December of 2019, pre-pandemic. No doubt in the COVID-19 environment, companies likely sought even more assistance from providers to help guide them in the altered world of person-to-person contact.



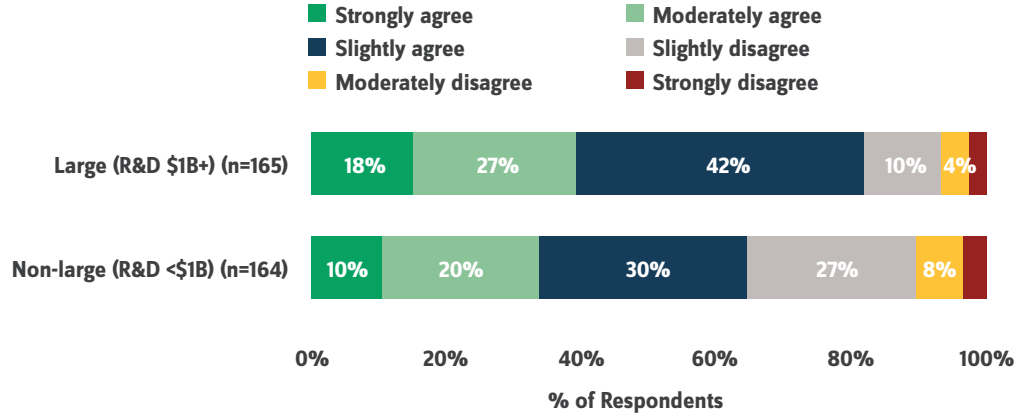
When considering company size, respondents at large (R&D $1B+) pharma companies have a greater expectation regarding these future plans compared to respondents at non-large (R&D less than $1B) pharma. In terms of increasing the use of specialized patient recruitment companies over the next two years, only 17 percent of respondents at large companies disagreed that this will be the case, compared to 31 percent of respondents — nearly double — at non-large pharma. Shedding light on this pain point, one dissatisfied respondent commented, "We are using [Provider] for a study in a difficult population in a difficult country and their local expertise has not resulted in timely study start or other activities."

MY ORGANIZATION WILL INCREASE THE USE OF SPECIALIZED PATIENT RECRUITMENT COMPANIES OVER THE NEXT TWO YEARS.



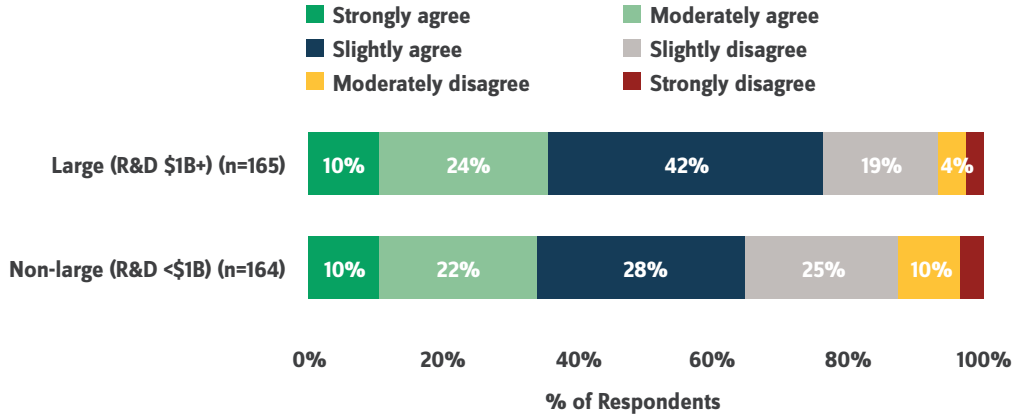
Similarly, more than 85 percent of respondents at large pharma companies agreed that their clinical trial designs plan to enroll diverse populations, compared to 60 percent of respondents at non-large pharma. One satisfied respondent gave this comment with their provider rating, "[Provider] has strong regional regulatory intelligence, site networks, etc., that allow for rapid start-up in AsiaPac."

CLINICAL TRIAL DESIGNS AT MY COMPANY
ARE FOCUSING ON ENROLLING A DEMOGRAPHICALLY DIVERSE POPULATION



In terms of increasing the use of specialized site identification companies, the expectation is greater for respondents at large pharma companies compared to non-large pharma, 76 percent versus 60 percent, respectively. Several respondents said that specialized outside consultants assist their companies with this activity. One respondent shared that consultants provide "identification and qualification of potential sites, management of clinical sites, and clinical trial agreements." Another respondent specifically commented, "Finding high-recruiting academic sites with special patient populations."

MY ORGANIZATION WILL INCREASE THE USE
OF SPECIALIZED SITE IDENTIFICATION COMPANIES OVER THE NEXT TWO YEARS



Indeed, if "show me the patients" wasn't already being uttered (hollered?) across this industry, it is now for multiple reasons. Of course, patient access and patient recruitment are by no means new challenges for the sponsors facing them and the providers accommodating them. The new challenge ahead is navigating these pain points during present times. Companies pre-pandemic were already looking to providers for help. After all, improving the human condition doesn't stop because the sky falls. But, it will require some heavy cloud lifting. ISR

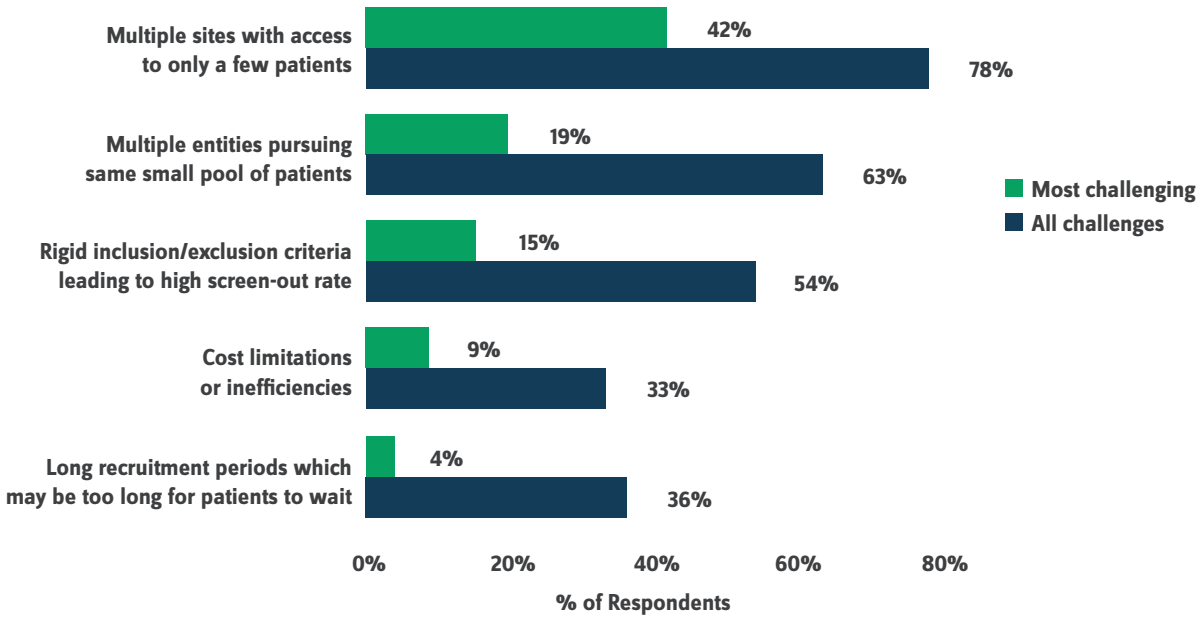
Top 5 Challenges In Finding Rare Disease Patients

INDUSTRY STANDARD RESEARCH

ISR asked respondents with involvement in patient recruitment and retention activities for Phase II/III rare disease studies about their pain points regarding patient recruitment. By far, the most challenging issue reported by respondents in finding patients for their recent rare disease studies was multiple sites with access to only a few patients (42%). The second most challenging issue was multiple entities pursuing the same small pool of patients (19%), followed by rigid inclusion/exclusion criteria leading to high screen-out rates (15%).

"What challenges do you experience in finding patients for your Phase II/III rare disease studies? Select all that apply." (n=125)

"Of those you selected, which is the most challenging when finding patients? Select one." (n=125)



"Why is 'multiple sites with access to only a few patients' the most challenging when finding patients?" (open end)

"Even when considering patient-friendly protocol, strong feasibility, early capturing of data analytics, country selection, and knowledge of strong study sites — having fewer patients within the rare disease space places greater pressure on knowing where those patients are and picking sites with good access to them and/or having access via decentralized or virtual efforts."

"Once you've recruited larger institutions with high patient throughput (which has its own challenges, e.g., patients come from long distances for treatment and don't want to return for follow-up visits), you're left with many sites that have the potential to provide only a few patients." ISR

Rare Disease Studies: A Comfortable Patient Is A Retained Patient

SHERRY HUBBARD-BEDNASZ Market Research Director, Industry Standard Research

What is comfort anyway? Being home. Your own bed. Your mom's homemade anything. In rare disease studies, those things are suspended. Get through the trial and then you can seek comfort. But does it have to be this way?

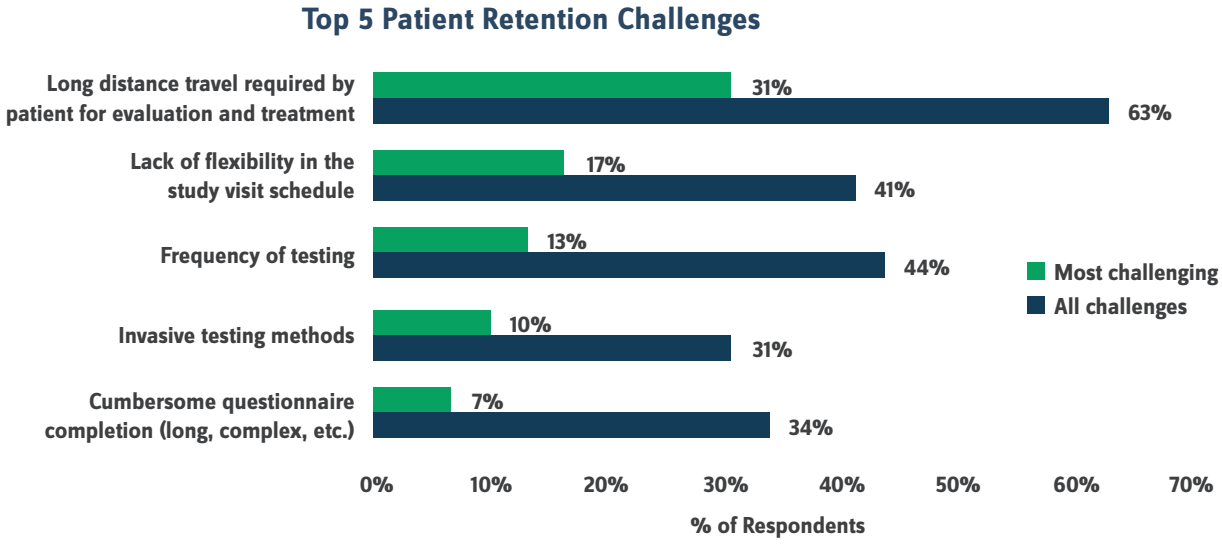
One could say the cards are stacked against rare disease studies. We often talk about the challenges of clinical trials in the frame of typical studies – drug development efforts intended to improve the condition of many. The key word is many. Rare diseases do not have numbers on their side. This is not to say typical studies are cake walks. There are no guarantees with human trials. And definitely not in the midst of a global pandemic.

But rare disease studies are special. And deservedly so. Greater challenges are baked into rare disease studies compared to typical studies: very small patient pools, specialized protocols, difficult-to-define endpoints, often very sick patients with limited ability or time, and the predicament of high cost versus low volume. These things undoubtedly create barriers to success from the word go.

Ironically, more people suffer from a rare disease than you might think. The NIH speculates that 25 million to 30 million Americans are living with any of the estimated 7,000 rare diseases known to exist. Let's recap: lots of people with lots of unique conditions; huge unmet needs for the drug development industry; pharma companies, big and small, expressing interest. A trifecta of possibility, right? In the world of rare disease, there is no easy button, no magic wand, and no jetpacks.

Industry Standard Research wanted to better understand the nuances associated with rare disease studies. Specifically, what does the uphill battle of patient retention look like? What are the secret weapons to minimize conditions conducive to dropout? In a study conducted in April of 2020, 125 industry professionals involved in rare disease patient recruitment and retention activities were candid about their experiences. No clinical trial guru is needed to tell you that very sick patients have limits to what they can do in the name of science. But does that mean protocols are embracing patient comfort as much as they should?

First, we had to gather the obstacles. We asked respondents to pick from a long list of potential challenges they face regarding patient retention. We then asked which one was the most challenging. Long distance travel, lack of flexibility in visit schedule, and testing frequency were cited the most. The greatest challenge, Long distance travel required by patient for evaluation and treatment, was selected by one-third of respondents.



One respondent did not mince words about the burden of travel – both on patients and the study. "We fly patients from Brazil, Argentina, Chile, United Arab Emirates, and South Korea to the U.S. for clinical study participation. It's expensive, risky for patients who are ill, requires caregivers to take time off work, and right now (COVID-19), forbidden." Another respondent shared the dilemma that school-age patients face: "This is mainly a pediatric indication and the sufferers are often in a lot of discomfort so travelling is difficult. Also, the frequent long journey interferes with school."

For rare disease studies, travel is the beast to slay. As the world continues to stretch its capacity to stay connected in a non-physical way, the drug development industry is no different. We talked about COVID-19 being the unexpected agent of change to allow clinical trials to bend while still preserving their scientific rigor. Even in April, very early in the health crisis, one-third of respondents said their study protocols were flexible – locations such as the patient's home or doctor's office (non-site) in addition to the clinical setting could be used for data collection. Perhaps a juggling act for the study coordinator yet affords the patient a sense of control and relief.

We asked the respondents who used multiple data collection locations about specific outcomes. Of that group, the most cited outcome by 93% of respondents was Less travel for patients. Let's say that again – nearly all respondents said using multiple locations reduced travel. Increased patient compliance and Less patient dropout were the next most cited outcomes, garnering 60% of respondents each. Blow out the candles and your wish for study success just may be granted. Well, with a little support, that is...

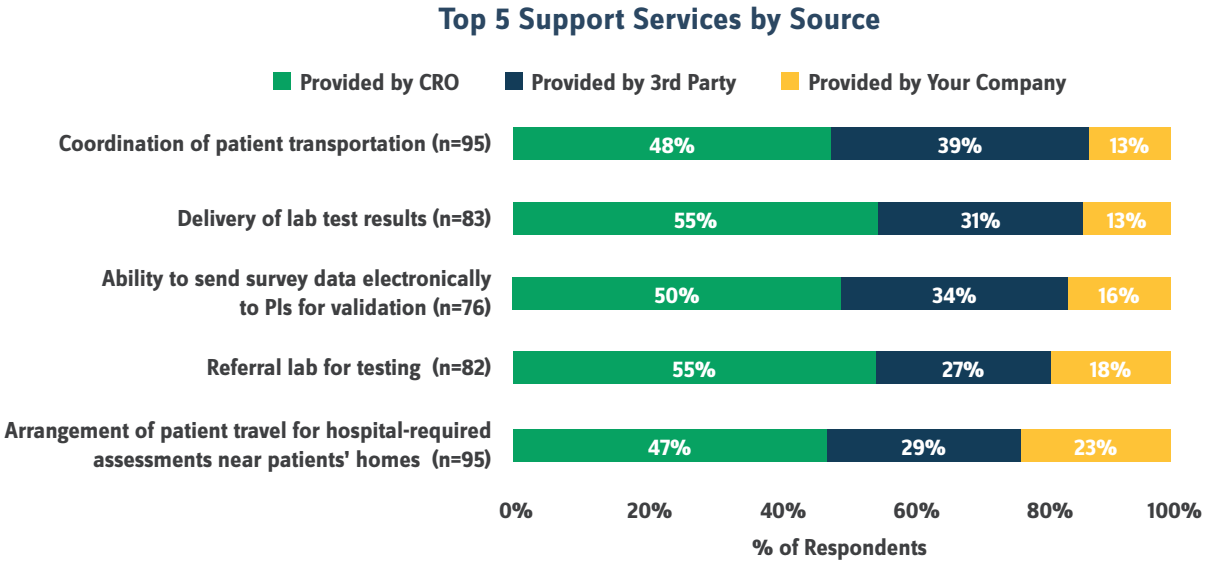
One respondent's candor paints a vivid picture of how protocol flexibility was a game changer for patient retention in his study: "Patient or their family members don't have to travel; they can provide the same information remotely and having multiple locations saves travel time. Patient is not subject to the inconvenience of long travel, spending time at the site, lodging, food, and other expenses, which add to emotional and mental agony."

Indeed, allowing rare disease patients to flex where and how they give study data is an invaluable gesture of fitting the trial to the patient, not the patient to the trial. Sick patients would travel 3 hours twice a week for eight weeks for an exhaustive battery of tests IF they could. But many can't. In-home data collection brings the clinical setting to them. We asked respondents specifically about their use of the in-home clinical trial support model, which takes into account personal preferences of patients, their disease symptoms, and their difficulties in travelling long distances to sites. About one-quarter of respondents said they used this patient-centric model in their recent studies.

When asked about the impact of using the in-home support model, most respondents shared the same sentiment – the model kept patients in their studies. One respondent reflected on the balance between patient control and study compliance: "Decreasing the patient's burden regarding travel and work/school time loss increases the retention significantly due to the in-home support. The patients still feel confident that they are followed up and monitored, what is indeed the case."

Back to that little mention of support. Conducting a trial where much of the activity is happening outside of the clinical setting requires some interplay of third-party support. Clinical staff alone often cannot accommodate the myriad of logistics that trial flexibility requires. We asked respondents about their use of support services – everything from patient transportation to referral labs to delivery of refrigerated meds. In these times, service providers are reassessing their offerings to meet the needs of the new normal. Whether we call these trials open, virtual, decentralized, or flat out different, they come with quite a few moving pieces and parts.

Support services that respondents used most follow this running theme of minimizing patient travel. Services related to transporting patients landed at the top – Coordination of patient transportation (81%) and Arrangement of patient travel for hospital-required assessments near patients' homes (79%). Referral lab for testing and Delivery of lab test results followed with two-thirds of respondents each. Knowing the type of support service needed is step one. Knowing who offers the service – and performs well – is a whole other Google search. The chart below shows the top five utilized support services by source. About half of respondents said support services were CRO-provided and about one-third said they were third party-provided.



In our world of market research, we aim to reduce respondent burden. Yes, of course we want to ask respondents a hundred questions on any given topic and collect all the data imaginable. But we don't. Respondent fatigue is a real thing, reliability is never guaranteed, and we want respondents to stay engaged through the end. No, our studies are not clinical trials. And no, our respondents are typically not too sick to participate. The parallel, however, is this – rare disease studies can better minimize burden, give some flexibility, and offer a little comfort to patients to stay in the potentially life saving game. ISR

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Smarter questions ∴ Smarter answers

Six Questions to Ask About Your Market Research

How do you guarantee the research you buy will give you confidence in your decisions? Here's how we ensure you're getting the value you should expect from quality market research.

How Many Participants Take the Study?
Understanding your margin of error gives you accurate expectations, making you more likely to hit your performance metrics. We provide a vast sample of participants from our proprietary Health Panel to make sure our studies reach the correct number of participants needed for accurate analysis.

Where Did the Participants Come From?
Eliminating sample bias translates into accurate competitive information and improves service quality by ensuring your decisions are the right ones. Our Health Panel provides an array of participants from all company sizes within the pharmaceutical industry.

When Were the Data Collected?
This should be the first question you receive during a presentation and saying "I don't know" doesn't sound so good. In all our research products, we collect up-to-date data relevant to the project at hand.

Who Sponsored the Research?
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Nothing stops a presentation faster than management questioning the basis of your research. Confidently project the research knowing that we pull information from key decision makers.

What is the Background of the Analyst Who Managed the Project and Reporting?
We have experienced analysts with hands-on industry knowledge. Their insights can quickly be turned into fit-for-purpose recommendations for your organization.