

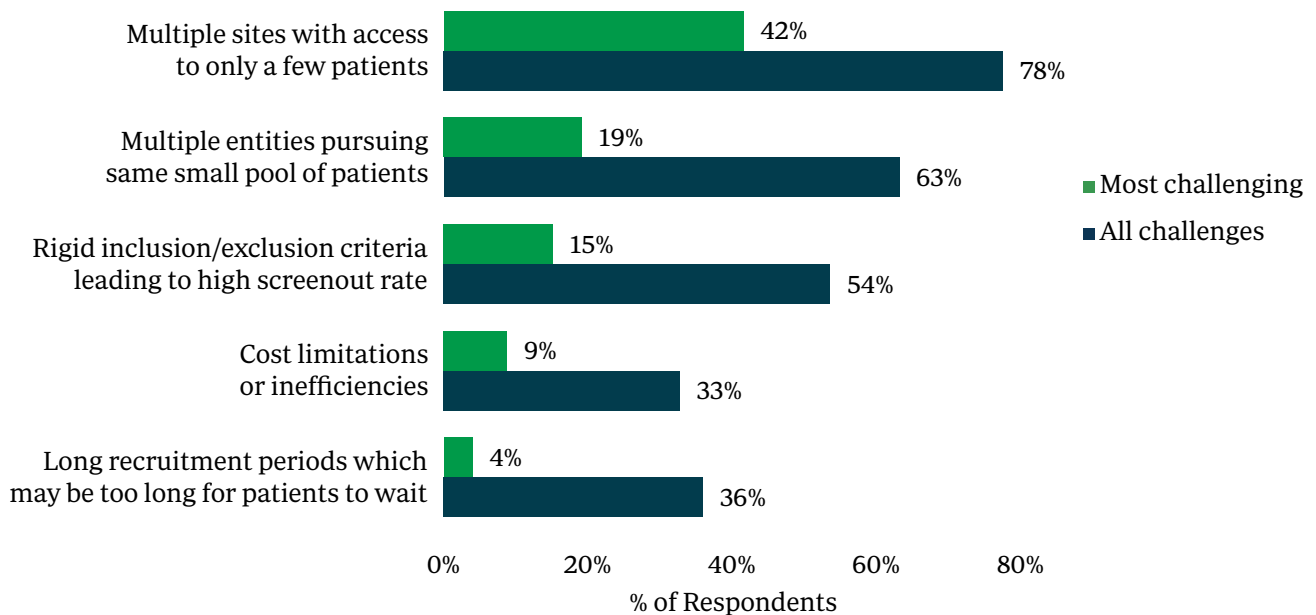
# Top Five Challenges In Finding Rare Disease Patients



ISR asked respondents with involvement in patient recruitment and retention activities for Phase 2/3 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 same small pool of patients* (19%), followed by *Rigid inclusion/exclusion criteria leading to high screenout rate* (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.”