Not Like Everyone Else: Unique Challenges for Small Biopharma Companies

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While "biopharma" is often referred to as a single category, the actual diversity of biopharma companies is often under-recognized by those outside the industry. Small and emerging biopharma companies are conducting almost 50% of the clinical trials in the United States today.[1] Unlike "big pharma", small companies are usually working with lean teams, limited resources, and minimal infrastructure- or are trying to build an infrastructure and manage ongoing clinical trials at the same time. When product pipelines are small, there is significant internal and external attention on every study and every milestone. Working in rare disease indications with small populations also brings new challenges to clinical study design and recruitment planning. These conditions create unique challenges for the clinical development teams within these companies. In this paper, we explore some of those challenges, and solutions that can reduce the pressure on teams and timelines.

1. Working with lean teams

Part of the fun of working in small biopharma companies, and especially very small companies, is that no matter how someone's specific role is defined, they can end up doing a variety of things. As part of the clinical team at small companies, I've drafted press releases, participated in investor meetings, watched market research focus groups, and conducted due diligence on possible business partners. It's exciting because there are new things to learn and new things to do—but at the same time, it means that the clinical team is always stretched in multiple directions.

It's also true that no matter how experienced a team may be, new situations may call for additional expertise. The clinical development of a gene therapy product is different from a small molecule product with regard to regulatory oversight, safety considerations, and informed consent requirements. Specific federal regulations related to research in children that differ from the regulations for adults may surprise sponsors. Pulling in consultants in specialty areas to provide support, assistance with regulatory submissions, or to provide training for sponsor clinical teams and even for site staff can make a huge difference in being prepared for the unexpected and staying on timelines.

2. Rare Diseases are rare, and special

While it's not always the case that small biopharma companies are working in rare, or ultrarare, disease indications, this is often true. Product development in rare diseases is fundamentally different from developing products for more common indications. While many companies are making efforts to improve the patient-centricity of their clinical programs, companies working in rare diseases have been leading the way in this area for decades, forging close collaborations with patients and patient advocacy communities.

Rare diseases also bring clinical trial challenges. The selection of clinical sites for studies is a critical decision; opening sites that will have little or no enrollment wastes time and resources. Having the data to make these decisions is essential. Sites will often be academic medical centers which are notoriously slow to move protocols through the contract and approval process, and often short on resources and attention to enrollment after the initial burst of enthusiasm. Look for partners who can help with study start-up; providing comprehensive data to make site selection decisions, and are experienced in contract and budget negotiations to maximize your enrollment period. Once the study is underway, specialized services like Enrollment Assistants can supplement the site staff under the direction of the investigator to ensure that there is continuing attention to study screening and enrollment; even when the site team is being pulled in multiple directions.

3. Finding the right-sized partners

It's also often true that finding partners for clinical trial services and support with experience in a rare disease, or similar clinical settings, means working with large companies with an extensive profile of therapeutic area experience. But for small biopharma, working with large partners is sometimes not the best fit. Many small biopharma sponsors are rethinking their outsourcing models and moving away from "one vendor does it all" and toward a collaborative system of niche product and service providers who are right-sized for the sponsor. These specialty partners are usually purpose-built; that is, they were founded with the intention of being the best at providing a small number of specific services. And the niche partners are usually very experienced at working with—and sometimes even within—multiple CROs, so they can integrate into project teams easily.

4. Be the Change!

The technologies that have evolved to streamline the administration of clinical trials are extensive and impressive. We now have online site feasibility surveys with real-time visibility into results, fully electronic essential document collection, online site team training and learning management systems, electronic distribution of investigator safety letters with automated tracking of review and central monitoring, and many more products that make our old manual processes so much easier. However, many

of these technologies have been slow to be adopted into practice, and study site start-up timelines aren't improving,[2] in large part because big companies are hesitant to move away from "the way we've always

done it here at XY Biopharma."

Small companies are in a great position to take advantage of these time-saving technologies, bring them in early and scale them up as the pipeline grows and products advance through development. This is the time to partner with someone to develop electronic Patient Reporting Outcome (ePRO) instruments, put your mandatory GCP and protocol training online, to set up an electronic Trial Master File with automated essential document collection, and to look for other time-saving technologies. Be open to partners who can create solutions for you and don't be afraid to try something new.

5. Don't build what you don't need (yet)

And along with looking for innovative solutions, don't get farther ahead than you need to on your infrastructure build. For several years, I was a medical consultant to biopharma companies, and many of my clients were small companies who needed physician review and oversight of certain activities, but didn't need someone full-time yet. But in these same companies, I'd sometimes see one or two divisions built out far ahead of what was needed at the time- like a clinical safety group with 3 full-time team members for a product that was receiving only a few SAE reports each month. Rather than building teams and investing in expensive software licenses, consider outsourcing parts of the infrastructure to partners who can manage that function until you're ready to bring it back in house (or, until you have a development partner who will). For example, a partner can provide all your safety report receipt, processing, report writing, software license and letter distribution functions, scaling the support to be as much or as little as you need. Rather than asking multiple CROs to take this function on for individual studies and ending up with multiple separate safety databases that need to be combined, you'll have one product-wide solution. A partner can manage and facilitate all the site contract and budget negotiations between your company and the clinical sites, taking burden off your legal team.

Conclusions

Small and emerging biopharma companies will always have unique challenges—wbut there's help available. Small companies are in a great position to adopt new technologies, take advantage of the services that partners can provide, and to leave behind the "that's the way we've always done it" mindset that has prevented the clinical trials operations field from moving forward at the same speed as medical advances.

About the Author

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References

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