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When it comes to small populations, biotechs need to think big

Maria-Cruz Morillo, global therapeutic operations lead for rare diseases at Allucent, discusses the unique challenges of rare disease drug development and how the right expertise can help

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By Allucent

The challenges biotechnology companies face in developing new medicines are increasingly dynamic and complex—particularly in rare disease clinical research, where patient populations for some conditions may be less than 25 people worldwide. Now, more than ever, companies need to have the right expertise in place to remain competitive and meet the unique demands of this area of research.

Maria-Cruz Morillo is focused on helping companies "get it right" as the global therapeutic operations lead for rare diseases at the specialized Clinical Research Organization (CRO) leader, Allucent. Allucent helps bring new therapies to light by solving some of the distinct challenges faced by small and mid-sized biotech companies in the drug development journey.

How did you get involved in clinical research, and what makes you passionate about this space?

I spent the first part of my career in clinical development at biotechnology and pharmaceutical companies, and the last 15 years working in the CRO setting. I've seen in my own family how difficult facing a rare disease can be, and the possibility to deliver hope to people in such a vulnerable position is so rewarding. Working in drug development for rare diseases is so exciting because we have opportunities to bring new medicines to patients who currently have limited or no treatment options.

What do companies need to consider when designing a rare disease clinical trial to be truly patient-centric?

Rare disease research needs to address three key challenges: the difficulty of identifying the patients, accommodating their specific needs and lessening their burden to participate. Close collaboration with patients, advocates and families on these topics is critical—we need their voices.

If there can only be a very small number of clinical trial sites, we need to consider if the people in our trial are able to travel. What accommodations are needed? Minimizing the number of visits to the clinical site by contracting home nurse services for capturing certain data, or using remote monitoring of patient data such as through a wearable device, may help.

Another important factor is how trial measurement criteria are defined. These should include not only the presence of certain biomarkers but also measures that are meaningful to the patients and their families, such as whether their loved one can now walk or read.

What should biotech companies look for in a partner or a team helping with clinical trial design and execution in rare diseases?

Specific expertise and a tailored approach are needed. Many of our regulatory and biometrics consultants previously worked for global regulatory authorities and understand exactly what will be required during the journey to approval. Technical data management staff are experienced in analyzing patient-reported outcomes and smaller, complex data sets.

With deep knowledge and experience to "go big" in thinking of the small details, the right plan can be developed to address complex challenges and guide the way from strategy through approval. We focus on tailored solutions, flexibility and patient-centricity, including a dedicated decentralized clinical trial approach called Patient Direct that incorporates real-world data collection, remote monitoring and other technologies to broaden our reach and make participation easier for patients and their families.

What opportunities in rare disease drug development are you most excited about, as you look ahead to the future?

The growth in genetic testing capabilities, not only of patients but family members, will yield larger datasets of mutations that can in turn advance the diagnosis and treatment of rare diseases.

The growing use of Real-World Evidence (RWE) is also very exciting. Particularly when patient numbers are limited, using real-world data for external control arms could help avoid the need to give patients in the trial a placebo while supporting the statistical significance of the data.

I also believe we'll continue to see more decentralized and direct-to-patient solutions for capturing data remotely.

These are three areas that offer important opportunities to address some of the challenges that come with rare disease drug development. And, it's really exciting to see, because a lot of great work is already underway. With the addition of more data, if we can make this available across all stakeholders—clinicians, academia, pharmacies, patients and advocates—I believe we will see even more progress ahead. So, I'm very excited about the future and extremely hopeful for what can be accomplished to help bring needed treatments to people with rare diseases around the world.