ERGOMED
Clinical Research



Case Study

Bridging the Gap: A Sponsor's Path to Meaningful PAO Engagement

Introduction

The Role of PAO in Rare Disease Research

Patient advocacy organizations (PAOs) play a crucial role in the healthcare ecosystem, particularly in rare diseases where patient communities are inherently small and often underrepresented and underserved. These organizations serve as the trusted voice of the communities they represent, raising awareness of the medical condition, amplifying patient voices, providing support and education, ensuring access to clinical trials and approved therapies, and advocating for policies to drive development of innovative treatments.

Pharmaceutical companies and PAOs share a common mission of improving the lives of patients, creating a natural foundation for collaboration. However, establishing these relationships requires careful navigation, especially when a sponsor is new to a therapeutic area or not yet connected to a patient community.

Challenges and Opportunities

When a pharmaceutical company has no existing relationship with a patient community, they may face significant challenges in developing therapies and conducting clinical trials that truly address the needs of patients, care network and families affected by the medical condition. Here, we examine how Ergomed facilitates relationship-building between a sponsor and a rare disease PAO.

In this case, the sponsor had:

- A new clinical trial planned in the US
- A small footprint in the US market
- No previous experience in the specific disease space
- No established relationship with the relevant patient community

The objective was to establish a meaningful connection between the sponsor and an appropriate PAO to ensure that the design and execution of the clinical trial would be ethically sound, patient-centered, and positioned for success.

Our Approach: A Thoughtful, Patient-Centric Engagement Framework

Establishing ethical and effective relationships with PAOs requires a thoughtful, systematic approach guided by the following best practices:

1. **Understanding PAO operations**, including organizational mechanics and rules of engagement.
2. **Assessing the patient landscape** to gain a thorough understanding of the patient community.
3. **Researching the PAO**, including its mission, vision, audience, campaigns, and programs.
4. **Initiating transparent communication with the PAO** to identify shared objectives and opportunities for collaboration.
5. **Providing financial support** to help strengthen the mission and activities of the PAO.
6. **Building trust** with ongoing nurturing to maintain and develop the relationship over time.

Step-by-Step Execution

Ergomed's active role in educating the sponsor on the principles of engagement with an appropriate PAO involved a stepwise process:

1. **Identifying potential PAOs** by researching and selecting those organizations most relevant to the disease state under investigation.
2. **Performing initial outreach** to assess PAO interest.
3. **Facilitating the introduction** between the sponsor and the PAO.
4. **Reviewing the clinical trial**, including the scientific justification and goals of the study.
5. **Gathering insights** from the PAO regarding the clinical trial approach and the culture of the patient community.
6. **Identifying common goals** and opportunities for the sponsor to support the PAO.
7. **Establishing a communication framework** with channels for open and ongoing communication.

Real-World Impact and Application

Having established a relationship with a relevant PAO, the sponsor gained the opportunity to meet and speak directly to patients through joint sponsor-PAO initiatives such as in-person meetings and fundraising events, thus forging genuine connections with the community they aimed to serve. By listening to the patient voice and journey, the sponsor was able to apply input from the patient community into the study protocol and to identify strategies for overcoming participation barriers and increasing diversity.

This collaborative relationship also supported the development of patient-centric educational materials that addressed the questions and concerns most relevant to the community, written in accessible language. In addition, the sponsor was able to participate in clinician-focused meetings that helped to bridge the gap between scientific objectives and study feasibility.

Throughout this process, regular follow-up and consistent communication maintained the momentum of the partnership, ensuring that the patient perspective remained central to the clinical development continuum.

This sustained engagement transformed what could have been a transactional relationship into a true partnership that furthered the common mission of both the sponsor and the PAO.

Results and Key Takeaways

For sponsors seeking to engage with PAOs, focusing on what is most important to patients, their care network, and their families ensures ethical, responsible, and meaningful interaction with the PAO.

In this case, close collaboration with a relevant rare disease PAO led to improved clinical trial awareness, recruitment, and retention. Continued communication with PAO afforded the sponsor access to ongoing insight and guidance throughout study conduct, fortifying trust and leading to an enduring relationship with not just the PAO and the patient community, but clinicians as well.

Thus, the value of PAO engagement extended beyond the individual trial to support ongoing development and eventual therapeutic access.

Ready to build lasting relationships with rare disease communities? Connect with our patient engagement experts today.

Therapeutic Expertise, Ergo, Informed Trial Strategies

At Ergomed, we don't just run clinical trials – we redefine them. With our industry expertise, cutting-edge solutions, and unwavering commitment to patient-centered research, we empower clients to achieve success in even the most complex studies.

Let's build the future of clinical research, together.

ERGOMED Clinical Research

ergomedcro.com