Worldwide Clinical Trials

Guide

# Engaging with Rare Oncology Research Consortia:

## Forging Meaningful Relationships


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Rare oncology diseases have historically presented substantial challenges. However, with the increased push toward engaging with specialized research consortia, the field is shifting toward more targeted and effective clinical interventions. These consortia, collaborative networks of clinical sites and expert leaders, address the unique challenges of rare cancer research, from protocol design to patient recruitment. By shifting the focus from a trial-centric to a patient-centric approach, consortia help to enhance the scientific rigor of clinical trials and ensure that the patient voice is heard. As the landscape of cancer care continues to move toward patient-reported outcomes (PROs) and overall well-being, consortia have become increasingly vital, particularly when engaged for clinical trials in tandem with patient advocacy groups (PAGs).

## Rare Oncology Research Consortia: The Patients' Scientific Voice

Rare oncology research consortia are collaborative networks that enhance knowledge and treatment options for less prevalent cancers. Although each of these cancers is rare in isolation, collectively they constitute a substantial portion of the oncology spectrum. The intricacies of rare oncology research are diverse, from the inception of clinical protocols to the location of suitable trial sites and the recruitment of participants. Each phase of this process encounters distinct challenges, primarily due to the infrequency and diversity of the diseases under investigation.[1,2] For example, the limited pool of patients with a specific rare cancer can render the recruitment of an adequate sample size for meaningful clinical data quite challenging. Moreover, the low volume of patients complicates identifying and accessing sites with the requisite expertise and patient populations.

> The approach to rare oncology research has evolved from a trial-centric to a more patient-centric model.

Consortia fill a critical gap between passion and scientific and clinical expertise.

The shift to a patient-centric model reflects the understanding that clinical trials' efficacy is contingent on patients' effective engagement and support. PAGs have been pivotal in this transformation, often originating from a small cohort directly affected by the disease.[3] These groups generate passion and commitment, working tirelessly to raise awareness, advocate for improved care, and support patients and their families. They are often the first point of contact for patients and their families and provide information about available treatments and clinical trials.


**Figure 1.** An effective rare oncology intervention requires collaboration and active cross-talk between key entities. Partnering with an experienced CRO can help the sponsor successfully navigate the complicated clinical development pathway.

While PAGs excel in advocacy and engagement, they may lack the extensive clinical and scientific expertise necessary for the design and execution of complex clinical trials. This gap is where consortia play a crucial role. Consortia, comprised of various clinical sites and expert medical and scientific leaders, bring a wealth of knowledge and resources to the process. They uniquely position themselves to drive policy changes and facilitate the successful operation of clinical trials, ensuring that the research remains rigorous and relevant.[4]

In the context of the increasing prominence of precision medicine in oncology, the role of consortia in protocol design and trial operations is of the utmost importance. Oncologists increasingly use precision medicine to treat rare oncology patients, emphasizing individualized treatment based on genetic and molecular profiles, and necessitating a more targeted approach to patient recruitment.[5,6] Consortia can facilitate identifying and accessing appropriate patient populations through their extensive networks and expertise. This targeted approach is essential for the development of therapies that are efficacious for specific patient subgroups. Importantly, the collaboration between consortia and PAGs is often synergistic, with patient-centric insights informing the scientific and clinical strategies.

## Consortia-Led Trial Designs: The New Normal

Consortia-led trial designs are the new standard in rare cancer research, offering the potential for more streamlined and effective outcomes by consolidating resources and expertise. The unique characteristics of rare cancers present considerable obstacles to traditional clinical trial frameworks. The scarcity of patients and the specialized care required for these conditions can impede the ability of individual research centers to conduct comprehensive investigations. Consortia, however, provide a platform for the collaboration of expert networks, key opinion leaders (KOLs), and clinicians who collectively assess clinical trial protocols, thereby ensuring a rigorous and well-informed scientific approach. This cooperative model expedites the research process and heightens the prospects of success by capitalizing on its participants' varied experiences and insights.

Consortia are essentially knowledge hubs focused on the operational and medical components of clinical trials. They ensure that researchers conduct trials with the highest scientific rigor, which is critical for the patients' success.

When PAGs and consortia work together, they create a powerful synergy that benefits the entire rare cancer community.

The influence of consortia extends beyond the realm of trial design to that of policy development and community engagement. By pooling their collective experience and expertise, consortia can be powerful advocates for changes in healthcare policies that support research and care for patients with rare cancers. Their capacity to engage a broad spectrum of stakeholders, from researchers to regulatory agencies, ensures that the unique needs of these patients are not marginalized.[7] Furthermore, consortia are pivotal in raising public awareness about the significance of rare cancer research, which is essential for securing funding and public support.

## Historic Perspective

The significance of the present-day consortia model is best understood in the context of the historical challenges that have led to its development. In the early 1950s, the National Cancer Institute (NCI) identified a research void concerning rare cancers.[8] These diseases, often marginalized in terms of funding and investigation, were hindered by a lack of resources and collaborative networks necessary for scientific progress. The disjointed nature of research and the limited financial support available to individual institutions underscored the need for a more cohesive and coordinated approach.

The NCI's response was the establishment of the Cooperative Group Program, which brought together ten independent groups to collectively address the complexities of cancer research.[9] Each group had its unique network of clinical sites, infrastructure, and scientific expertise, but the program had limitations. The need for more direct and integrated collaboration became apparent as the cancer research landscape evolved. The Cooperative Group Program was a significant advancement, but it became evident that a new model was necessary to address the specific challenges of oncology, particularly in the context of rare cancers.

This recognition led to the reorganization of the NCI and the establishment of the National Clinical Trial Network (NCTN) in 2014.[10] The NCTN represents a shift in the operation of research consortia and their contributions to the oncology field. In contrast to the previous model, the NCTN is a collaborative network of organizations and clinicians that provides a robust infrastructure for NCI-funded research. This new structure was designed to facilitate more direct collaboration, enabling the effective implementation and study of the latest advances in precision medicine and cancer care.

The transition from the Cooperative Group Program to the NCTN is emblematic of a broader evolution in the role of research consortia. Originally conceived as networks of clinical sites, these entities facilitated access to patient populations and multi-site trials. However, the passive nature of these networks was ill-suited to the demands of rare oncology research. Consortia, as they are now, have adopted a more proactive stance, emphasizing the incorporation of patient perspectives, the rigorous conduct of trials, and the application of specialized expertise. These features are particularly pertinent in rare oncology, where identifying and validating biomarkers and testing novel interventions are often complex and require a precision medicine approach.

The transformation into more integrated and patient-centric entities has improved the quality and relevance of clinical trials and positioned them for greater success in obtaining FDA approval. By establishing new guidelines and standards of care, the NCTN and associated consortia have played a pivotal role in advancing the field of rare oncology. The increased emphasis on patient engagement and the adoption of precision medicine principles have been instrumental in this evolution, driving the consortia to become more than just a means of accessing patients and sites; they are now key players in shaping the future of rare cancer research and treatment.

## Effective Engagement Creates Added Value

Effective engagement among consortia, researchers, and patients is more than courtesy; it is a crucial component that significantly enhances the research process. When patients contribute to the design of clinical trials, the studies become more pertinent. This collaboration ensures that the trials address the most critical concerns and needs of those who will ultimately benefit from the research. For example, patients can offer insights into the practical aspects of trial participation, such as the feasibility of traveling to trial sites or the acceptability of specific interventions. These insights can assist researchers in designing trials that are more likely to succeed and yield actionable results.

Patient involvement can lead to higher recruitment rates, better adherence to study protocols, and more meaningful outcomes.

By convening a network of experts, consortia can offer invaluable input on trial designs and protocols, thereby enhancing the quality and feasibility of the research. Site access, for example, is often predicated on the consortium's approval of the trial design and proposed intervention, ensuring a well-structured trial with a greater likelihood of achieving its objectives. Furthermore, collaboration with consortia as part of the research team can lead to direct and impactful outcomes, such as changes in clinical guidelines and the potential to redefine standards of care, to benefit current and future patients.

Advanced trial designs and cooperative efforts from consortia optimize research for success before regulatory approval. Consortia can identify potential pitfalls and suggest improvements, rendering the trial more robust and credible. Real-world evidence collected through these collaborations is highly valuable, providing a more extensive and varied dataset that informs policy and practice. Engaging with consortia also provides access to global expertise across multiple sites, which is particularly important in the swiftly changing field of oncology research. This international perspective informs researchers about current and future regulatory guidance and mandates, ensuring their work aligns with the latest standards and requirements, which is essential for obtaining the necessary approvals and implementing research findings in clinical settings.

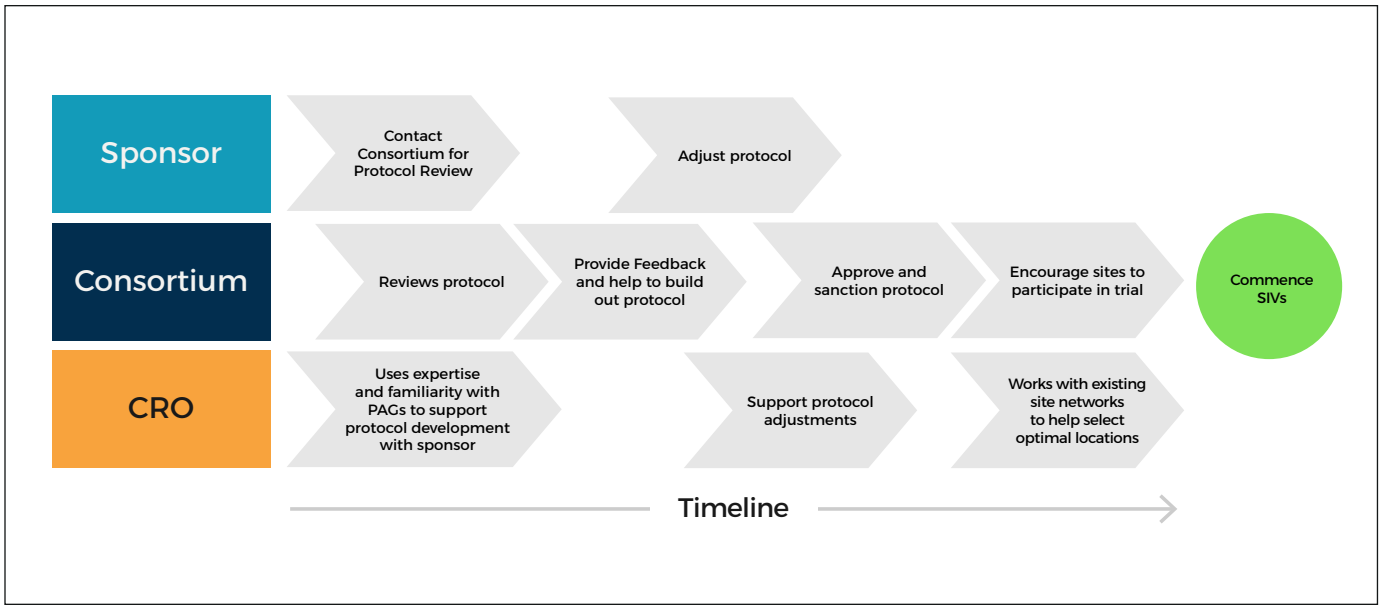


**Figure 2**. An example flowchart for effective consortium engagement for a rare oncology trial. In some cases, the consortium will assist with site selection, contract negotiations, and budget. Selecting a CRO with previous experience and established relationships with both PAGs and consortia in the rare indication ensures your protocol has the necessary factors in place for expedited protocol approval and site initiation visits (SIVs). This style of engagement facilitates a protocol that keeps the patient voice and scientific rigor at the forefront, ultimately speeding up the clinical trial timeline and ensuring participant retention.

## Future Directions

The future of rare oncology research will involve increased collaboration alongside technological advancements, which may provide hope for patients with rare cancers. One of the most important changes is the increased emphasis on PROs and reducing treatment-related toxicities. Researchers and clinicians are beginning to appreciate that maintaining or improving patients' quality of life is as important as prolonging survival.[11] By incorporating PROs into clinical trial designs, consortia can better understand the real-world impact of treatments, which may lead to better treatment options.

Precision medicine is rapidly becoming the standard in oncology, and consortia are pivotal in this transformation. These collaborative networks provide unparalleled access to the right sites, laboratories, principal investigators, and patient populations. By leveraging the expertise and resources of consortia, researchers can more effectively identify and validate biomarkers, leading to more personalized treatment options. This approach enhances the efficacy of treatments and reduces the risk of adverse effects, further improving patient outcomes.

The involvement of KOLs is another crucial aspect in advancing rare oncology research. KOLs are often at the vanguard of emerging technologies and therapeutics, and their perspectives are invaluable in shaping the trajectory of clinical trials and research endeavors. Consortia provide a platform for these experts to exchange knowledge and collaborate, thereby expediting the development of innovative treatments and diagnostic methodologies.

The expansion of rare cancer patient registries is on the horizon, promising researchers a more comprehensive and diverse pool of clinical data. These registries are pivotal in elucidating rare cancer patients' distinct characteristics and requirements, facilitating more precise and efficacious clinical trials. Moreover, their expansion is expected to contribute to discovering new biomarkers, indispensable for tailoring personalized treatment strategies.

Developing novel biomarkers is a critical step for future progress in rare oncology.[12] Biomarkers can predict treatment responses, identify high-risk patients, and inform the development of more efficacious therapies.[13,14] As biomarker research advances, it is anticipated that more tailored treatment options will emerge, addressing the specific genetic and molecular profiles of rare cancers.[15] These advancements further underscore consortia's critical role in facilitating scientifically sound and patient-forward protocols. This is especially true because a personalized approach can enhance patient outcomes and significantly decrease the impact of disease.

The success of these trials and research initiatives may have significant implications, potentially influence new guidelines, and improve care for conditions with unmet medical needs. By focusing on rare cancers, consortia can generate insights that benefit a broader spectrum of patients, fostering a more comprehensive and integrated approach to oncology. The collective efforts of consortia, PAGs, and KOLs are crucial in transforming the landscape of cancer care, bringing hope and better outcomes to those in need.

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