Worldwide Clinical Trials

The Value of Hope in Oncology Clinical Trials

Michael F. Murphy, MD, PhD | Chief Medical and Scientific Officer

Matthew Confeld, PharmD, PhD | Associate Director, Clinical Research Methodology

Michael Perfetti, PhD | Clinical Trial Liaison - Global Oncology

Article | May 2025

The foundation of differentiated CRO services comprises the integration of scientific acumen, medical expertise, and predictable, repeatable trial operations. However, woven into the fabric of this business philosophy is a belief that all CRO services, regardless of therapeutic area, treatment modality, or phase of research, are intended to facilitate the introduction of novel therapeutics to patients with considerable unmet needs. The pharmaceutical industry collaboratively develops a narrative that exemplifies the value of hope embodied in treatment through participation in industry-sponsored interventional clinical research.

Contingent on the details of protocol design an interventional study may attempt to capture patient-specific outcomes, perform a priori or post-hoc subgroup analyses to identify those patients more likely to respond, conduct exit interviews at study conclusions to contextualize treatment outcomes, or explore clinically important covariates of treatment response to shape the population in which therapeutic benefit might be more readily discerned. The ability to engage in comprehensive exploratory clinical research at earlier phases of drug development using assessments that reflect how patients "feel and function" acknowledges that the hope for a cure, or remediation of debilitating symptoms is a prime determinant of patient participation in clinical research.

Oncology is emblematic of the challenges associated with evaluation of innovative pharmacotherapy in the context of significant unmet clinical needs. Despite remarkable advances in recent years that have resulted in innovative therapeutics increasingly targeted toward highly nuanced patient populations, many cancers — especially rare or aggressive types — still lack effective treatments. Patients with advanced disease often exhaust standard therapies and are left with limited options for care, underscoring a pressing need for new approaches. The therapeutic landscape is additionally complicated by significant attrition in clinical development, attributed to several different drug discovery and development limitations inherent in the context of a disease with exceptional heterogeneity in terms of molecular targets and clinical phenotypes.[1]

The Multiple Dimensions of Hope

The motivation for study participation in clinical research generally aggregates into nine domains, (Table 1) with each domain providing a different dimension in which the "value of hope" can be operationalized.

Access to innovative therapy is a prime motivating factor for patient participation in the face of a shortened lifespan. Even in the face of low success rates, the mere possibility of a breakthrough can be a powerful motivator for patient and family participation. Patients often see trials as a chance for a novel treatment to either enhance survival or reduce morbidity, while also providing a sense of purpose and contribution to a greater cause.

Table 1

Motivation Type	Example
Access to new treatments	*"I want to try the latest therapy not available elsewhere."*
Personal health benefit	*"I hope this trial will help me live longer or feel better."*
Trust in doctor and trial team	*"I trust my oncologist's recommendation to join this study."*
Enhanced monitoring and care	*"I like the idea of being closely watched and cared for during the trial."*
Altruism	*"Even if it doesn't help me, maybe it will help others with cancer."*
Hope and coping	*"Participating gives me hope and something to fight for."*
Family influence	*"My family wants me to try every possible option."*
Financial or logistical factors	*"The trial covers some costs, making it easier for me to get treatment."*
Information-seeking	*"I want to learn more about my condition and what treatments are out there."*

However, patients may also take an altruistic stance, hoping their participation can help those in the future. Similarly, these trials likely present an opportunity to try something innovative and potentially useful, a uniformly voiced sentiment in structured patient interviews.[2] Even if the trial does not directly benefit them, the knowledge that they are part of something larger can provide a sense of fulfillment and purpose. In this context, hope is not just for the patients of today, but also the patients of tomorrow and the future.

Hope also resides in the trial experience itself. Patients often find comfort in the heightened monitoring and access to world-renowned disease experts. This level of care and attention can make patients feel valued and supported, providing a sense of safe passage through a challenging journey. For others, trial participation offers the potential to spend more quality time with family and loved ones by delaying the time to deterioration, maintaining overall quality of life (QoL), and allowing for a sense of normalcy and connection with their support network. The emotional and psychological benefits of this cannot be overstated, as they can provide a much-needed boost in morale and well-being.

> "I want to be here as long as I can with my wife, kids, and the life we have been blessed with. I want to have good quality of life during my treatment and that God will give me strength and peace during course of my journey."
>
> – Kevin Calvert

Informing Access to Treatment

The high costs of medical treatments can be a crushing burden and enrolling in a trial can provide a financial reprieve. The financial support offered by many trials can alleviate substantial economic stress, making it a more viable option for those who might otherwise be unable to afford the necessary treatments. Indeed, the American Society Of Clinical Oncology Value Framework and the European Society For Medical Oncology Magnitude Of Clinical Benefit Scales, attempt to incorporate and summarize a comprehensive assessment of value differing in the emphasis placed upon QoL, toxicity, efficacy or economic sequelae, as well as their intended use (e.g., shared decision-making or input on policy and reimbursement).[3]

Prioritizing the Patient and Caregiver Voice

Family or caregivers can be crucial in a patient's decision to join a trial. The encouragement and presence of loved ones provides emotional strength to navigate the potential uncertainties that may arise during study participation. This support system can make the difference between a patient that is feeling isolated and overwhelmed to one who feels empowered and supported.

Caregivers often deeply understand the patient's needs and can provide valuable insights into the potential benefits and risks of trial participation. Their role in advocating for the patient and navigating the complexities of the healthcare system can be invaluable, making the trial experience more manageable and less daunting.

It is the patients and caregivers that comprise the heart of every clinical trial, whose voices are key to meaningful insights. These insights reach beyond anecdotes; they are vital for ensuring that the trial remains relevant and practical, better serving the needs of those directly impacted. When designing a clinical trial, keeping the patient and those affected by the condition at the forefront is essential. By doing so, researchers can create protocols that are better aligned with the real-world experiences and needs of the participants.

The growing importance of patient-reported outcomes within oncological research exemplifies the emphasis on patient and caregiver assessments associated with study participation. This focus is particularly relevant for those illnesses characterized by a more extended lifespan that, in some cases, might approximate those of individuals who are not afflicted. These constructs include multiple domains spanning disease symptoms, cognition, physical functioning, and social and emotional well-being. These outcomes are essential for building a value proposition that supports formulary placement and reimbursement policies, as an example, that may largely be associated with the perceived value of the drug.[4]

Collaborative design, where patients and caregivers are involved in the planning and execution of the trial, can significantly improve adherence and outcomes. Methods include the use of structure partnership models, patient advisory panels, and caregiver informed consent processes with iterative feedback loops in which families and patients can review draft protocols for modifications.[5] Patients who feel that their input is valued are more likely to remain engaged and committed, which leads to more accurate and reliable data. Indeed, trials using co-design strategies report higher enrollment rates, and procedures and interventions aligned more closely with patients' priorities maximize retention. Moreover, involving caregivers in the design process ensures that the trial accounts for the broader impact on the family unit, which is often overlooked but critically and equally important. Thus, collaborative designs ultimately enhance the relevance of all outcomes for the patient and the family.

Real-world data from patient diaries provides invaluable insights that traditional clinical data cannot. These diaries capture patients' day-to-day experiences, offering a more comprehensive understanding of how the treatment affects their lives. For instance, a patient might report that while a new treatment may not completely halt disease progression, it decreases the symptomatic burden and allows them to spend more quality time with their loved ones. This kind of data can be quantified using a technique known as the time trade-off method, which helps researchers understand the value of the treatment from the patient's perspective. With these real-world insights, researchers can better tailor treatments to improve overall QoL alongside other meaningful clinical outcomes.

Support groups provide a uniquely empowering opportunity for patients and caregivers, fostering a sense of community and shared experience. These groups provide a platform for individuals to share their stories, exchange advice, and offer mutual support. This sense of community can be a powerful motivator, helping participants stay committed to the trial even when faced with challenges. Additionally, support groups can serve as a valuable resource for researchers, providing feedback and insights to inform trial design and improve patient care. Through prioritizing the voices of patients and caregivers, clinical trials can metaphorically shift from being industry-sponsored to becoming patient-centered, leading to more meaningful and impactful results.

A Path Forward

The future of medical research centers on a collaborative approach, placing the primacy on scientific rigor, informed clinical care, and human compassion. Hope and the "value" that various stakeholders append to this concept, actively influences trial outcomes and personal well-being with novel pharmacotherapy. The ability to conduct exploratory research in early phase studies within oncology facilitates a systematic evaluation of drug effect, parsing effects across patient phenotypes, and outcomes, thus optimizing treatment strategies by adding dimensions for hypothesis generation or testing under the umbrella of "feels, functions, or survives."

In this process, patient and caregiver insights are invaluable in shaping more effective and compassionate clinical trial designs. Incorporating these key perspectives into study design and operational solutions creates a portfolio of studies that are more relevant and responsive to the needs of those they aim to help, ensuring that these treatments improve the day-to-day lives of those who rely upon them.

The authors would like to acknowledge the contribution of Jim Eamma, MSc, in the preparation of this white paper.

Contact us to continue the conversation in the context of your current or future oncology research efforts.

References

1. Kuo YM, Barrett JS. Consideration of the Root Causes in Candidate Attrition During Oncology Drug Development. Clin Pharmacol Drug Dev. 2024 Sep;13(9):952-960. PMID: 39162188. doi: 10.1002/cpdd.1464.
2. Wegge-Larsen C, Mehlsen M, Jensen AB. The Motivation of Breast Cancer Patients to Participate in a National Randomized Control Trial. Support Care Cancer. 2023 Jul 15;31(8):468. PMID: 37452876. PMCID: PMC10349774. doi: 10.1007/s00520-023-07930-0.
3. Bentley TGK, Cohen JT, Elkin EB, Huynh J, Mukherjea A, Neville TH, Mei M, Copher R, Knoth R, Popescu I, Lee J, Zambrano JM, Broder MS. Measuring the Value Of New Drugs: Validity and Reliability of 4 Value Assessment Frameworks in the Oncology Setting. J Manag Care Spec Pharm. 2017 Jun;23(6-a Suppl):S34-S48. PMID: 28535104. PMCID: PMC10585824. doi: 10.18553/jmcp.2017.23.6-a.s34.
4. Confeld M, Murphy MF. When a Lethal Disease Becomes Chronic: The Emergence of PROs in Oncology. Worldwide Clinical Trials. White Paper. Mar 2024. https://www.worldwide.com/insights/pros-in-oncology-white-paper/.
5. Seifart C, Koch M, Leppin N, Nagelschmidt K, Knorrenschild JR, Timmesfeld N, Rief W, von Blanckenburg P. Collaborative Advance Care Planning in Advanced Cancer Patients: Col-ACP -Study - Study Protocol of a Randomised Controlled Trial. BMC Palliat Care. 2020 Aug 24;19(1):134. PMID: 32838763. PMCID: PMC7445911. doi: 10.1186/s12904-020-00629-7.

20260603