Informed Consent: A Clinical Trials Perspective

Marilyn J. Hammer, PhD, DC, RN, Patricia Eckardt, PhD, RN, and Margaret Barton-Burke, PhD, RN, FAAN

The primary goal of the thousands of registered trials in cancer research is to extend survival. With evaluation of efficacy, safety, and tolerability, healthcare providers must ensure that the principles described in the Belmont Report are upheld and that patients are truly informed when signing a consent form. In this article, two cases are highlighted, and reasons for participating in clinical trials are discussed. Challenges, such as healthcare literacy, patients’ dedication to their healthcare providers, and choosing between multiple trials, are also explored.

More than 5,500 registered clinical trials exist that are researching cancer (ClinicalTrials.gov, 2016). With the primary goal of survival, clinical trials to test the efficacy, safety, and tolerability of pharmacologic agents are the gold standard (Ordering et al., 2016). For many patients with a poor prognosis, these trials can be extremely appealing. However, many patients are hesitant to participate. A 2010 clinical research workshop summary noted a number of barriers to enrolling in clinical trials, including fears about quality-of-life alterations, the possibility of receiving the placebo instead of the drug, side effects, the new drug potentially not being the best treatment, inconveniences in being part of the study, feeling coerced, wanting the physician to make the decision, and feeling a loss of control (English, Lebovitz, & Giffin, 2010). However, clinical trials give hope when standard therapies fail to help a patient’s disease.

Deciding to enroll in a clinical trial, in most cases, is an informed gamble. In many cases, the treatment advancements may be minimal at best. For example, a study evaluating survival, safety, and prognostic factors for the use of regorafenib (Stivarga®) in patients with metastatic colorectal cancer refractory to standard therapies boasted a median survival rate of 5.6 months, with 80% of patients experiencing at least one adverse event (Adenis et al., 2016). This type of outcome suggests the need to assess patient preferences related to sacrificing quality of life for the potential of a few extra months of life. Such decisions are difficult, but the glimpse of hope for an actual cure—no matter how small the chance—can be extremely enticing. Participating in the trial as the only means to receive the treatment may far outweigh contributing to scientific advancement for future patients. Of note, patients at any stage often enroll for altruistic reasons. Some patients have expressed their appreciation for research participants who enrolled in studies prior to their own diagnoses and advanced the scientific knowledge that now gives them the opportunity for prolonged survival.

For clinical trials and all research studies, reviewing the Belmont Report’s focus on ensuring respect for persons, beneficence, and justice is essential (U.S. Department of Health and Human Services, 1979). Examining two publicly highlighted cases, the current article will explore the application of these ethical tenets for patients who signed informed consent forms to receive gene therapy.

Case Study of a Patient With an Enzyme Deficiency

The first case involves an 18-year-old man, Jesse Gelsinger, with a rare immunologic disease in which his body lacked an enzyme, ornithine transcarbamylase, needed to