Case 4a: Randomized Controlled Trials of Bone Marrow Transplants for the Treatment of Advanced Breast Cancer in Women

An increasing number of women with advanced breast cancer are convinced that a bone marrow transplant, which involves a very high dose of chemotherapy followed by the transplant, is the best treatment option. In many cases, their doctors agree. The effectiveness of this treatment for advanced breast cancer has not been conclusively demonstrated, however.

The National Cancer Institute in the United States is sponsoring three large randomized trials to determine whether bone marrow transplants are preferable to the chemotherapy regimens that are now standard treatment for advanced breast cancer. In these trials, about half of the participants are randomly allocated to receive the transplant and the balance receives the standard treatment. Many women with advanced breast cancer are turning to bone marrow transplants outside of scientific trials, however. In view of poor survival rates associated with conventional treatments, they are unwilling to take the chance they will not be assigned to the experimental group. As a result, researchers are having difficulty enrolling enough participants in the trials. Without proper studies, there is a danger that no one will ever know whether the transplants are actually preferable to conventional treatments.

The transplants are grueling and risky for the breast cancer patients. Roughly 5 percent of women who undergo a bone marrow transplant die as a result of the treatment. The transplants are also expensive. Whereas conventional chemotherapy costs $5,000 to $25,000, bone marrow transplants run from $60,000 to $200,000. In response to lawsuits, an increasing number of insurance companies have agreed to pay for the transplants.

Questions for Discussion
1. In 1994 alone, more than 1,000 women with breast cancer underwent bone marrow transplants in the United States outside of clinical trials. Would it be ethical to require patients to enroll in a clinical trial in order to gain access to this experimental therapy?
2. What would be the risks and potential benefits if this expensive new treatment were to become the treatment-of-choice for advanced breast cancer, with or without adequate evidence from randomized clinical trials?
3. Is it ethical for a physician investigator to enroll his or her patients in a randomized trial of bone marrow transplants if he/she is fairly sure, but not absolutely sure, that the transplants are better than conventional therapy?
4. Clinical researchers often have a stake in the timely completion of clinical trials. In those situations where physicians have a financial or professional incentive to maximize the number of their patients enrolled in a trial to what extent does this represent conflicting interests?

Reference