Chapter 24
Cancer:
Clinical Trial Design and Principles

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ABSTRACT

Clinical trials are essential to govern the impact of a new possible treatment. It is utilized to determine the safety level and efficacy of a certain treatment. Clinical trial studies in cancer have provided successful treatment leading to longer survival span in the patients. The design of clinical trials for cancer has been done to find new ways to prevent, diagnose, treat, and manage symptoms of the disease. This chapter will provide detailed information on different aspects of clinical trials in cancer research. Protocols outlining the design and method to conduct a clinical trial in each phase will be discussed. The process and the conditions applied in each phase (I, II, and III) will be described precisely. The design of trials done in every aspect such as prevention, immunochemotherapy, diagnosis, and treatment to combat cancer will be illustrated. Also, recent innovations in clinical design strategies and principles behind it as well as the use of recent advances in artificial intelligence in reshaping key steps of clinical trial design to increase trial success rates.

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INTRODUCTION

A continuous increase in the global burden of cancer is primarily because of the factors like world population, pollution, food habits, aging, and adaptation of certain cancer-causing behaviors in developing countries. Based on GLOBOSCAN estimates in 2018, 18.1 million new cases of cancer were registered and 9.6 million deaths occurred worldwide. One in 6 women and one in 5 men during their lifetime develop cancer. Also, one in 11 women and one in 8 men die from cancer. (Latest global cancer data 2018) It has become a leading cause of death among people in economically developed countries second leading cause among people of developing countries. This makes cancer research and its drug discovery more prevalent than other diseases.

Clinical trials are essential to govern the impact of a new possible drug or treatment. Clinical trials are utilized to determine the safety level and efficacy of a certain drug or treatment. Clinical trial studies in cancer have provided successful treatments leading to longer survival span of the patients. The design of clinical trials for cancer has been done to explore new ways to prevent, diagnose, treat, and manage symptoms of the disease. In the mid-1950s, the first randomized clinical trial was carried out and within two decades it was proved as a useful way to detect the relative effectiveness of cancer treatments and substantial progress has been made to date. The clinicians and statisticians should cooperatively put efforts in the design and analysis of studies as well as there should be a synergistic effort between clinicians and patients too during the conduction of such studies (Edmund A. G., 1979)

HOW IS A CLINICAL TRIAL FOR CANCER COMPARED TO OTHER THERAPEUTIC AREAS?

Oncology is found to be complicated than other therapeutic areas to an extent. One major difference is the endpoints; like in clinical trials for other therapeutics, the aim is to test the efficacy and safety of a certain antibiotic against infection whereas oncology clinical trials try to extend the quality of life of the subject. Also, there is a difference, in how the severities of the events have been reported; in non-oncology clinical trials, the events are considered as mild, moderate or severe whereas in oncology trials the adverse events are divided into numeric grades according to National Cancer Institute guidelines: Common Terminology Criteria for Adverse Events (CTCAE). In some cases, mild corresponds to grade 1, grade 2 corresponds to moderate, grade 3 corresponds to severe, and grade 4 corresponds to life-threatening condition and to death.

TYPES OF ONCOLOGY CLINICAL TRIAL

Prevention

The prevention trials focus on ways of preventing cancer or recurrence. For example, the trial focusing on the use of vitamins, change in diet, the use of different medications or exercise for decreasing the chances of developing cancer. A related study was done considering a hypothesis that if the intake of dietary fat is reduced, the incidence of breast cancer will also reduce. It was found that total dietary fiber was associated with lower breast cancer risk in the early adulthood of women (Maryam S.F. et al., 2016).