data developed through the course of the phase I studies are used to determine the doses that will be used in phase II and phase III clinical trials.

Phase II typically involves 100 to 300 patients and are designed to determine whether or not the clinical candidate provides the desired biological impact. Safety studies also continue through phase II trials. In the first part of phase II trials, referred to as phase IIA, the goal is to determine the dose required to provide the desired therapeutic impact or endpoint for the clinical candidate. Once the proper dose levels are determined, phase IIB studies can be initiated. The goal of phase IIB studies is to determine the overall efficacy of candidate compounds in a limited population of subjects. The majority of clinical drug candidates fail in phase II studies due to safety issues or lack of efficacy. As of 2011, only 34% of phase II clinical candidates successfully reach phase III studies.

The effectiveness of new drug candidates in larger patient population are determined in phase III clinical trial. These studies are typically randomized and involve hundreds to thousands of patients at multiple clinical trial sites and are designed to determine the efficacy of the candidate compound relative to the current standard of care. The cost and time associated with this phase of clinical study can vary dramatically depending on the clinical endpoint under investigation. Clinical trials for new, acute treatments, such as novel antibacterial agents, are shorter and involve far fewer patients than clinical trials for chronic conditions such as osteoarthritis. Patients are also closely monitored for adverse side effects, as the larger patient pools can identify safety issues that did not become apparent in smaller phase II trials. The number of subjects, time requirements, and complex design of phase III clinical trials (especially in chronic medical conditions) dictate that they are the most expensive aspect of drug discovery and development. Upon completion of phase III trials, a New Drug Application is submitted to the appropriate regulatory body. This document typically contains comprehensive details of both animal and human studies, all safety findings (adverse and side effects), manufacturing procedures (including methods of analysis to ensure drug quality), detailed formulation information for all dosing methods studied, and storage conditions. Regulatory reviews can lead to requests for additional information regarding the submission, or even additional clinical trials to further establish either safety or efficacy. Ideally, these reviews lead to regulatory approval, including labeling requirements, and approval to market the new drug.[38]

Approval of regulatory bodies does not, however, signal the end of clinical trials. In many cases, regulatory agencies will require additional follow-up studies, often referred to as phase IV trials or postmarketing surveillance. In general, these studies are designed to detect rare adverse effects across a much larger population of patients than could be supported in phase III trials or long-term adverse effects that might be outside of the scope of phase