One of the major concerns of those responsible for the conduct of biomedical research in the United States is how to overcome the "translation gap" which exists between laboratory discovery and a reasonably rapid application of that knowledge to the improved prevention, diagnosis, and treatment of disease. One way of bridging that gap is through the emerging methodology of the clinical trial. This form of clinical research dates back to the period just following the end of the Second World War and was developed for the most part in Great Britain through the efforts of Sir Bradford Hill. More recently, it has become an increasingly large factor in clinical research supported by the National Institutes of Health, including the National Eye Institute (NEI).1,2

Because clinical trials involve groups of patients rather than individuals, these studies must incorporate a particular methodology consisting of three essential elements: the selection of patients to participate must be carefully defined; assignment of patients to a control group must be made using random procedures; precautions must be taken to avoid investigator bias by single or double "masking," and the ethical basis for trying a new therapeutic modality must be established. The latter can be done only if the advantages and disadvantages of a new procedure appear to balance those of the old or of no treatment at all. When such conditions prevail, then the most ethical study design is to assign the patients randomly to receive either the new treatment, the conventional treatment, or no treatment. In this way, each patient has a 50:50 chance of receiving the treatment that ultimately will prove to be better.

The clinical trial follows the principles of the scientific method by establishing a working hypothesis, designing an experiment to test the hypothesis, evaluating carefully the results, and reaching an appropriate conclusion. It differs from laboratory research in that the clinical trial usually deals with large numbers of patients; is very expensive; utilizes extensive resources of manpower, equipment, and supplies; takes many years to complete; and often requires the collaboration of several medical facilities throughout the country, working together within a complex administrative framework. Nevertheless, this methodology is invaluable for translating a working hypothesis from the laboratory into an experiment in the clinic,
as well as providing a basis for bringing science into the art and practice of medicine.

An excellent example of the success of such a cooperative clinical trial has recently been reported. From information collected during a two-year period on about 1,750 patients, it is now possible to define those stages of diabetic retinopathy for which photocoagulation treatment is likely to be beneficial during this time. This study continues, but already is certain to be recorded as one of the most successful clinical trials in the history of medicine.

The NEI is committed to the expansion of clinical trials, funding them primarily through research contracts. Through this approach, the safety and effectiveness of major new ophthalmic therapeutic techniques will be carefully and scientifically evaluated and the American public can be assured that it will receive the very best care possible.

Carl Kupfer
Director, National Eye Institute
Department of Health, Education,
and Welfare
Public Health Service
National Institutes of Health
Bethesda, Md. 20014

REFERENCES