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L'essai thérapeutique chez l'homme

Second edition

Paris: Flammarion Medecine Sciences

and

Daniel Schwartz, Robert Flamant and Joseph Lellouch (1980)

Clinical Trials

(Translated by MJR Healy)

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Preamble

As the frontispiece states, this book was published originally in French as *L'essai thérapeutique chez l'homme* by Flammarion Medecine Sciences in 1970 (see no. (23) above).

Aims

This book is not a working tool. It is intended as a stimulant to thought and also, we hope, to discussion. The translation of the book into the language of those who have made such contributions to the birth and development of statistics opens up a new range of possibilities for discussion (Author's preface, page vii).

Advances in therapeutics have always been a central objective for the clinician, yet the means used to attain them have remained for a long time in the domain of empiricism, of clinical impressions, of the rather more-or-less. Needless to say, considerable progress has been achieved. Recognition of the first effective treatment for a serious disease say, of streptomycin in the case of tuberculosis meningitis, does not call for sophisticated techniques. But such triumphs are never common and they will become ever less so as the range of therapeutics extends. More usually, advances in therapeutics will come to consist of a succession of small improvements, even perhaps of the discovery of treatments which are not more efficacious but simply better tolerated by the patient. When such small differences are to be established only carefully conducted trials will be able to provide definite information. In order to make progress in therapeutics, it is necessary to try out new treatments. The novelty of a new treatment may relate to the nature of a drug or of a technique, it may be associated with its method of administration, or it may consist of a new way of combining known agents or techniques. A comparative trial may be undertaken with more than one aim in view. One type of objective is both simple and well-known – it is required to ascertain whether the new treatment actually possesses the favourable activity in man which laboratory studies have led us to expect. This is typical of the situation with a new drug when we wish to confirm its efficacy as an analgesic, an antibiotic agent or whatever. In this case, the clinical trial is a direct extension of laboratory experimentation and is motivated by the same research-oriented attitude. The other type of objective is often overlooked by scientists and is less well understood. We now wish to assess the practical value of a new treatment in relation to other existing treatments. Here again we need a comparative trial, but now we must take into account all the advantages and drawbacks of the various possible treatments with a view to making a recommendation for clinical practice. The trial is aimed at providing practising clinicians with a basis for decisions concerning the choice of therapy and its motivation is strictly practical in nature. We shall refer to the two approaches we have outlined as “explanatory” and “pragmatic”; both approaches involve the type of comparative clinical trial which forms the

main subject of this book, but the differences between them call for parallel differences in the methods adopted (Introduction, pages 3 to 5).

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