A Method of Assessing Efficacy with Small Patient Numbers

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The method presented in this paper will, in the author's opinion, serve to document clinical results when numbers are small. There is no need for controls, and documentation may also be retrospective.

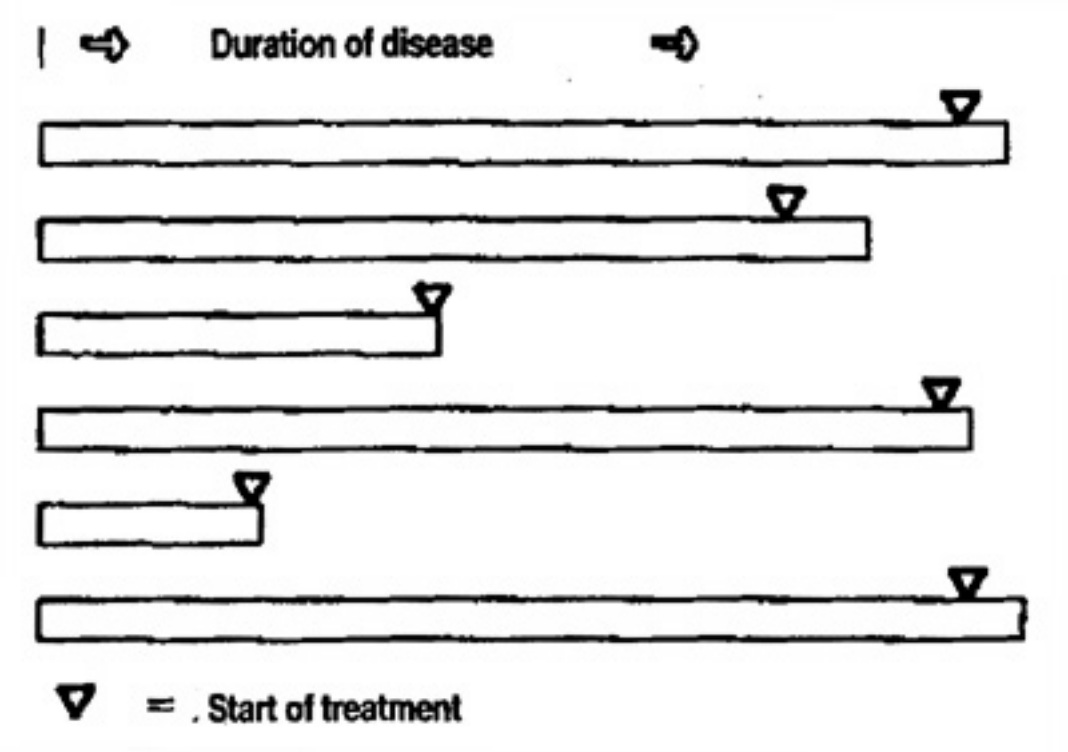
Essentially the method bases on a criterion often used in everyday clinical practice - the ratio of times for which symptoms persisted before and after treatment. If the time for which symptoms persisted after starting treatment is relatively short compared to the time before treatment, this may be taken to indicate that the treatment was successful. Results are most impressive if the post-treatment period comes close to zero. This is the kind of "instant cure" known from neural therapy, for instance. But when symptoms have persisted for five years, for example, and have disappeared four weeks after starting treatment, this, too, is a powerful indication of efficacy.

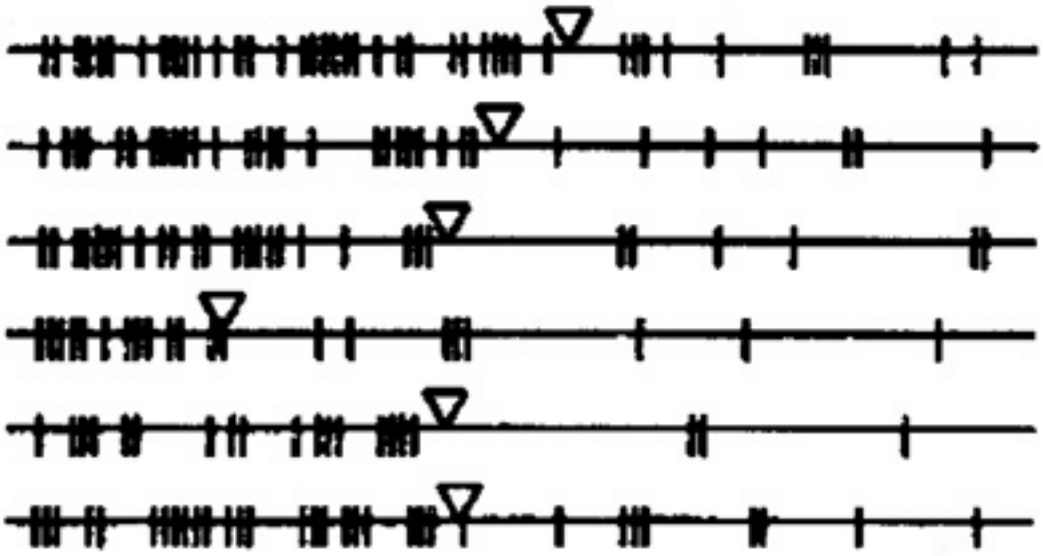
Such an indication may actually gain power of evidence if a similar situation can be shown for several patients and if this is also the total number of patients who have had the treatment in question. (This is the crux of the matter. There must be no selection of successful cases!) A highly convincing documentation of the efficacy of a treatment would be the following (unse-lected) complete set of six case records.

In this fictional example, let us say the period for which symptoms persisted after starting treatment was very short (reproduced in six consecutive cases) compared to the period before starting treatment. The argument that the improvements were spontaneous, happening by chance after treatment started, would not apply in this case, for it is highly improbable that the time of starting treatment and that of spontaneous recovery shortly after this would coincide like this on several occasions by sheer chance (six times in succession in this case). The probability of random coincidence decreases with the number of recorded cases. The more successful cases are recorded, the better.

(The objection that these are purely placebo effects can no longer be raised today unless it is properly founded. A study of the most recent literature shows that the existence of the placebo effect is very much in doubt. We might even ask if the existence of the placebo effect is not medical fiction.(1,2)

Graphics such as bar charts help us to present the clinical results in a convincing form. Such abstract diagrams ultimately provide the basis for overall assessment. Assessment is based on estimation, i.e. not on statistical analysis.


Duration of disease
= . Start of treatment


= Start of treatment
= Coughing attacks

The method cannot, of course, be used with all types of diseases and forms of treatment. It will not serve, for example, to demonstrate extension of survival for cancer patients. It may, however, provide effective documentary substantiation of a statement such as: "An ointment containing 10% of sea buckthorn seed oil... gives outstanding results... with the skin changes seen with neurodermatitis."(3)

A number of variations seem feasible. The following type of graphic presentation might be used, for example, to give a convincing demonstration that the frequency of whooping cough attacks decreased with a particular treatment.

Two aspects are of vital importance:

1. The total number of cases treated in a given context must be included. This calls for absolute faithfulness, otherwise the results will be falsified by selection bias.
2. The goal parameter must be clearly apparent. Clear criteria must exist to determine if the symptom in question is present or absent, and the degree of severity or otherwise of that symptom. Otherwise one easily gets observer bias.

The advantages of the method are as follows.

- The method requires relatively few cases. (The minimum would seem to be three. Power of evidence is, of course, all the greater the larger the number of cases showing a favorable ratio of pre- and post-treatment periods.)

- The method is flexible, i.e. it is possible to continue a set of records once it has been started, including cases that follow later.

- The method is robust, i.e. it can be used for retrospective studies. (Prospective studies do, of course, guarantee greater accuracy.) It is also possible to let a retrospective study continue on and become prospective.

- The method does not involve the ethical problems of randomized trials,(4) or the problematical subjective variations one gets when observing applications.

For all those reasons this is a method that may well suit practitioners who may otherwise find themselves excluded from research and documentation. It is important to note that so far no one has experience with the method (at least in the author's knowledge). There are bound to be some snags and problem areas that will only emerge when the method is put in practice. The form in which such documentation is published will play an inestimable role in its power to convince.

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