THE DRUG EFFICACY STUDY

Within the next 90 days, prescription labeling and promotional material on about 80% of currently prescribed drugs will display a rating of the drugs' efficacy for certain of the claimed indications.

The action is being taken by FDA in the belief that the prescribing physician must know the scientific status of a given drug's efficacy in order to exercise the best possible clinical judgment in choosing drugs for patients.

This section attempts to explain the aims and procedures of the National Academy of Sciences' Drug Efficacy Study (DESI) which led to this development.

BACKGROUND

The DESI program stems directly from requirements of the Federal Food, Drug, and Cosmetic Act. Beginning in 1938, this law required preclearance of new drugs by FDA for safety. The Drug Amendments of 1962 (Kefauver-Harris) required that effectiveness as well as safety of drugs be established prior to marketing. The amendment provided that this proof of efficacy be in the form of "substantial evidence." This evidence was defined by the Congress as ". . . adequate and well-controlled investigations, including clinical investigations, by experts qualified by scientific training and experience to evaluate the effectiveness of the drug involved, on the basis of which it could fairly and responsibly be concluded by such experts that the drug will have the effect it purports or is represented to have."

Therefore, since 1962, the FDA has reviewed all new drug applications for both safety and effectiveness. But the 1962 amendments also required that all drugs marketed between 1938 and 1962 and tested only for safety, now be evaluated for effectiveness as well.

Some 4,000 drug products fell into this category and efficacy evaluation for all of them obviously posed an enormous task.

To accomplish this task within a reasonable time, FDA went to the National Academy of Science for assistance. The Academy assembled 30 panels with some 200 medical and scientific specialists described in its 1969 report as "predominantly physicians with academic affiliations for the obvious reason that these best met the legal qualification of 'experts qualified by scientific training and experience to evaluate the effectiveness of the drug(s) involved.'"

The National Research Council — research arm of the National Academy of Sciences — developed guidelines for the study. In the course of its work, NRC consulted manufacturers, professional and scientific organizations and other interested parties.

The 30 study panels of the Academy considered information gathered from all these sources, including FDA files and the scientific literature. On the basis of this information, panel members were able to make informed judgments.

The panels classified each of approximately 16,000 therapeutic claims for the more than 4,000 drug formulations into the following categories:

Effective: substantial evidence of effectiveness;

Probably effective: additional evidence required to rate the drug "effective";

Possibly effective: while additional evidence for an "effective" rating might be forthcoming, as it stands there is little evidence of effectiveness, and in the absence of substantial evidence, the claim is considered inappropriate;

Ineffective: lack of substantial evidence of efficacy;

Ineffective as a fixed combination: even though one or more of the components might be effective if used alone, not acceptable in fixed dosage combination for reasons of safety or because of lack of evidence of contribution of each component to claimed effect;