CHAPTER

11

Introduction to Endpoints

I. FDA's GUIDANCE FOR INDUSTRY

Endpoints in clinical trials are used to assess the efficacy and safety of a drug or medical device. Clinical trial design typically includes primary endpoints, secondary endpoints, and exploratory endpoints. As explained by FDA's Guidance for Industry (1), clinical trial design includes a:

> hierarchy of endpoints ... determined by the trial's stated objectives and the clinical relevance and importance of each specific measure independently and in relationship to each other. We consider any endpoints that are not part of the prespecified hierarchy of primary and key secondary endpoints to be exploratory. Endpoints included for economic evaluation that are not intended for labeling claims should be designated as such, and will be regarded as exploratory.

Additionally, the FDA states that (2):

> primary and secondary efficacy endpoints should be chosen based on the drug's putative mechanism of action and the proposed indication ... [s]econdary efficacy endpoints can provide useful information on the effect of the treatment and ... provide support to the primary efficacy endpoint. Secondary efficacy endpoints also can explore other effects of the drug on the disease. Commonly used secondary efficacy endpoints include ... symptom scores, activity scales, and health-related quality-of-life instruments. Biomarkers can, in some cases, also provide support of efficacy.

FDA considers safety endpoints to be secondary endpoints (3). A publication from FDA officials provided a distinction between primary endpoints and secondary endpoints, "In considering approval or nonapproval of an application of a product ... the results from

[1]U.S. Department of Health and Human Services. Food and Drug Administration. Guidance for industry. Patient-reported outcome measures: use in medical product development to support labeling claim; December 2009 (39 pp.).

[2]U.S. Department of Health and Human Services. Food and Drug Administration. Guidance for industry. Chronic obstructive pulmonary disease: developing drugs for treatment; November 2009 (17 pp.).

[3]U.S. Department of Health and Human Services. Food and Drug Administration. Safety reporting requirements for INDs and BA/BE studies; December 2012 (29 pp.).

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