Statistical Guidelines

This section should be developed in close collaboration with the study biostatistician: at an early stage in protocol development.

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The outline below is modified from CTEP protocol templates for standard Phase I and II studies (http://ctep.info.nih.gov/protocolDevelopment/default.htm#protocol_development), and is meant to provide a rough guideline as to what information should be included, recognizing that this will depend on the particular protocol and design used.

- Describe the study design, which should include the goal(s) of the study (primary and secondary), the associated endpoint(s), and the method of dose escalation (e.g. "3+3"; accelerated titration; continual reassessment method (CRM)).
    - If the primary goal of the study is to identify a maximum tolerated dose (MTD) (or optimal biologic dose, or both) provide a precise definition of it or reference the appropriate protocol section.
    - If the study is randomized, indicate the proportion of subjects to be accrued to each dose level (e.g. equal randomization).
- Provide a statistical justification for the number of subjects to be enrolled based on the primary goal(s) of the study.
- If correlative studies are being performed, address the suitability of the proposed sample size to obtain meaningful results (even if the goal of such studies is hypothesis generating)
- Describe how the primary and secondary/correlative endpoints will be analyzed (e.g. parametric versus non-parametric methods; one versus two-sided statistical tests; the confidence limits that will be used for estimation).
    - If correlative studies are included, address whether or not adjustments to p-values will be made for multiple comparisons; and if appropriate the method to be used.
- Provide an estimate of the accrual rate.