CEA Study Evaluation Guidelines

Cost-Effectiveness Analysis (CEA) Studies Funding Program

Purpose and Background

As part of its Prioritization and Scientific Quality Initiatives, the Clinical Trials Working Group (CTWG) of NCI recommended establishing a funding mechanism and prioritization process for essential correlative biomarker, imaging, and QOL studies that are incorporated into the fundamental design of a clinical trial. In 2011, Cost Effectiveness Analysis (CEA) was added to this funding program. The objective is to ensure that the most important CEA studies can be conducted in a timely manner in association with NCI-sponsored clinical trials.

Cost-Effectiveness Analyses (CEA) provides useful information to help health care payers manage the use of costly medical technologies in order to maximize the health of their patient populations when facing constrained budgets, and to clinicians and patients to help guide treatment decisions based on CEA's unique endpoints, perspectives, and time horizon. To be most useful to decision-makers, CEA of new cancer therapies must have maximal feasibility, be timely, and have high internal validity.

Conducting a CEA alongside a clinical trial can achieve these goals and also offers the benefit of efficiency by utilizing the existing structure of clinical trials to collect additional data for the economic analysis. Support for timely and important studies during the clinical trial concept development phase will ensure timely development of effective, informative and high impact clinical trials.

The primary purpose of this funding mechanism is to support CEA studies that are paired with phase 3 clinical trials that have a comparator arm, conducted by the Cooperative Groups (CG's) and Community Cancer Oncology Program (CCOP) Research Bases.

Requirements and Definition

Eligible trial types are:

- Randomized Phase 3 clinical trial concepts with a comparator arm

CEA Studies

The CEA evaluation criteria are intended to help guide the selection of cancer clinical trials that warrant additional funds for a CEA. The CEA study should be a secondary endpoint of the parent concept. NCI's Scientific Steering Committees (SSCs) evaluate CEA proposals paired with clinical trial concepts, through their concept evaluation and prioritization process. SSCs will make use of ad hoc CEA expert(s), including resources available at the NCI, to evaluate CEA proposals included in clinical trial concepts.

Criteria for Review of CEA Proposals

Researchers should consider pairing a CEA proposal to phase 3 clinical trials when the following conditions are met:

- The results of a phase 3 clinical trial are expected to substantially influence clinical practice.

- The cost-effectiveness study would be of high impact judged by substantial budget implications for health care systems, either in terms of overall cost savings or added costs to the system.
- It is feasible to conduct a high quality CEA as part of the clinical trial. Specific issues to consider include:
  - The comparator arm should be relevant to current clinical practice.
  - The trial should be of sufficient duration, with respect to follow-up of patient outcomes, that consequences of interest to economic evaluation can be captured either directly or through modeling.
  - There is reasonable statistical power for the key cost-effectiveness analysis.
- Because of high cost, there is a reasonable degree of uncertainty regarding the outcome of the CEA even if the clinical outcome favors the experimental treatment.

CEA proposals included in phase 3 clinical trial concepts should be developed by CGs and CCOP Research Bases. When CGs and CCOP Research Bases choose to submit a CEA proposal, this must be submitted with the phase 3 parent clinical trial concept.

Cost-Effectiveness Analysis Proposal Package/Budget Submission

The BIQSFP CEA Proposal Package should include a completed *Study Checklist for Randomized Phase 3 Clinical Trials with a Comparator Arm and Cost-Effectiveness Analysis (CEA) Component* (see below). The CEA application should include a response to each of the elements. This section is not to exceed five (5) pages.

The CEA Proposal Package must also include a budget at the time of submission that clearly details the Direct and Indirect costs of the requested funding. The budget for the project should use the **BIQSFP Cost Estimate Worksheet** along with a narrative justifying each requested cost.

Study Checklist for Randomized Phase 3 Clinical Trials with a Comparator Arm and Cost-Effectiveness Analysis (CEA) Component

INSTRUCTIONS: Please submit a response to each of the criteria below and complete the BIQSFP Cost Estimate Worksheet.

1. Describe and justify the perspective of the CEA.

2. Explain the situations in which the outcomes of the clinical trial could substantially change clinical practice.

3. Describe the potential implication(s) of different outcomes of the trial on overall costs to the health care system, in terms of costs saved or costs added.

4. Briefly describe and justify the CEA study terms of:

   a) Trial population (in relationship to treatment population in community practice)

   b) Intervention(s) and control therapy selected for the CEA

   c) Question or hypothesis posed

   d) Measure(s) of outcome for the CEA

   e) Method of estimating costs

   f) Modeling approach proposed (if appropriate)

   g) Approach to characterizing uncertainty analysis

   h) The time horizon and discount rates of the CEA. If the time horizon of the CEA exceeds that of the trial, describe the extrapolation or modeling approach that will be used.

5. Describe any threats to the external validity of the study in relation to community practice.

As the CEA Reviewer, we ask that you please complete the attached CEA STUDY EVALUATION TEMPLATE.

Thank you.