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 Analysis (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 (e.g., societal, clinical, or third-party), and time horizon (e.g., within trial or long-term survivorship). To be most useful to decision-makers, CEA of new cancer therapies must have maximal feasibility, be timely, and have high internal validity. CEA funding may also apply to Symptom Science/Supportive Care clinical trials.

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. It is not required that a CEA proposal be included with each clinical trial concept submitted. However, in some instances the addition of CEA may be recommended during evaluation review of the clinical trial concept

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 must be a secondary endpoint of the parent concept. NCI Steering Committees (SC) evaluate CEA proposals paired with clinical trial concepts through their concept evaluation and prioritization process. NCI SCs will make use of an 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

Consider pairing a CEA proposal to phase 3 treatment or prevention clinical trials, or symptom science/supportive care clinical trials when the following conditions are met:

- The results of the clinical trial are expected to substantially influence clinical practice
- The cost-effectiveness study would be of high impact as 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 the follow-up of patient outcomes, that consequences of interest to economic evaluation can be captured either directly or through modeling.
  - There is sufficient statistical power for the key cost-effectiveness analysis
- Because of the high cost of the experimental treatment, there is a reasonable degree of uncertainty regarding the outcome of the CEA even if the clinical outcome favors the experimental treatment.
- Modeling is a crucial part of the CEA proposal. CEA proposals should describe the general type of model that will be used. If a model is to be developed, the expertise of the model developer, timeline for model development, calibration, and validation (if relevant) must be included in the proposal. This may include but not be limited to all model inputs that are needed as well as the respective sources for the inputs, what provisions are needed to document the model structure, assumptions, data inputs, parameter estimations as well as intermediate and final outputs so that replication of the CEA would be possible by an external analyst.

CEA proposals included in clinical trial concepts should be developed by NCTN/NCORPs. Anticipated/planned CEA studies should be noted on the respective CTEP/NCORP *Trial Concept Submission Form* and should be submitted **within three (3) months** of the PI receiving notification by the respective CTEP/DCP PIO, that the concept was approved.

It is not intended that any priority or particular level of merit is assigned to one criterion over another but rather the proposals are evaluated based on the totality of the information and strength of the data. Based on the strength of the information presented and your scientific judgment, you will be asked to rate your level of enthusiasm for the study on a five-point scale from High to Mild.

BIQSFP submissions should include a completed Study Checklist for each study. The completed Checklist should include a response to each element.

Please refer to the 2018 BIQSFP Guidelines *(https://www.cancer.gov/about-nci/organization/ccct/funding/biqsfp)* for additional program information.

Please complete and return the attached CEA EVALUATION TEMPLATE. Thank you.