

## QOL/PRO Study Evaluation Guidelines

### Quality of Life / Patient-Reported Outcomes Studies (QOL/PRO) 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 QOL studies that are incorporated into the fundamental design of a clinical trial. The objective of this initiative is to ensure that the most important quality of life studies can be initiated in a timely manner in association with clinical trials.

QOL/PRO studies embedded in clinical trials often lead to scientific observations that validate targets, reduce morbidity, predict treatment effectiveness, facilitate better drug design, identify populations that may better benefit from treatment, improve accrual and retention, and ultimately lead to change in the standard of practice. 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 QOL/PRO studies that are integral to and/or integrated with clinical treatment trials conducted by NCI National Clinical Trials Network (NCTN) groups and NCI Community Oncology Research Program (NCORP).

#### Quality of Life Studies

**QOL/PRO studies can be integral or integrated** assays, tests, and/or instruments. Studies may include biomarkers, imaging tests, PROs, or Cost Effectiveness Analysis (CEA). They must be part of the clinical trial design from the beginning (assessments conducted while the trial is open). They are intended to inform on treatment options and side effects by validating biological and functional clinical correlates.

Currently, DCP funds QOL/PRO components in disease treatment trials that obtain information for use in patient-physician decision making that help the patient prepare for and interpret the treatment experience, via DCP Cancer Control credits. Examples of this DCP support may include studies where differences between treatments in survival or other disease-related endpoints are expected to be minimal or when treatment arms represent very different treatment scenarios. Assessments may include, but are not limited to, qualitative data, toxicity impact, convenience, psychosocial outcomes and function.

**Integral Studies** - Defined as QOL/PRO studies that must be performed in order for the trial to proceed. Integral studies are inherent to the design of the trial from the onset and must be performed in real time for the conduct of the trial. Integral biomarkers associated with QOL/PRO studies require a CLIA-certified lab. Studies that will be conducted in the future on stored data are **not** eligible for BIQSFP funding, except if the results are critical to the stated primary or secondary objectives of the trial.

BIQSFP proposals for funding of integral QOL/PRO studies must be submitted concurrently with the parent concept.

**Integrated Studies** – Defined as assessments/tests that are clearly identified as part of the clinical trial from the beginning and are intended to identify or validate assessments/tests that are planned for use in future trials. Integrated studies in general should be designed to test a

hypothesis, not simply to generate a hypothesis. The number of integrated tests/assessments performed should be sufficient to obtain scientifically valid outcomes during the trial and include complete plans for data collection, measurements, proposed cutpoints, and statistical analysis.

**Anticipated/planned INTEGRATED QOL/PRO study applications** should be noted on the respective CTEP/NCORP Trial Concept Submission Form and must be submitted **within three (3) months** of the PI receiving notification by the respective CTEP/DCP PIO, that the concept was approved. Subsequent NCI prioritization and approval for funding will be decided by CTROC after evaluation of the QOL/PRO study by the respective NCI Steering Committee (SC), as applicable.

### **Criteria for Review and Prioritization of QOL/PRO Studies**

Prioritization and evaluation criteria include:

- The potential to impact patient morbidity and quality of life with clinically meaningful benefit.
- The potential to move science forward in cancer related symptom science/supportive care by adding critical knowledge.
- The strength of the preliminary data supporting the hypothesis(es) to be tested and methods proposed.
- A clearly defined process for data and specimen collection.
- A statistical plan with adequate power for testing the QOL/PRO correlative study hypothesis(es).
- Measures that are reliable, valid, and appropriate to the population of interest.
- Feasibility of the proposal such that completion can be accomplished efficiently and in a reasonable time frame.

Each category is of equal priority, however in general, higher consideration is placed on studies that are scientifically grounded and well developed, use well validated and reliable measures, and are likely to have the largest impact on clinical practice.

**It is not intended that any priority or particular level of merit be assigned to one of the previous criteria over another. 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.**

The BIQSFP submission should include a completed QOL/PRO Study Checklist for each QOL/PRO component. The elements in the QOL/PRO Study Checklist are listed below. The application should include a response to these elements.

### **'17 Study Checklist for Clinical Trials with QOL/PRO Endpoints**

**INSTRUCTIONS:** Please submit a response to each of the criteria below and complete one Study Checklist for each QOL/PRO endpoint. The 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 standard PHS 398 budget form (<http://grants.nih.gov/grants/funding/phs398/phs398.html>) along with a narrative justifying each requested cost. The Budget packet must include a completed NIH biosketch form for each study Principal Investigator (PI). Form SF424 can be found at: <http://grants.nih.gov/grants/funding/424/index.htm#format>. Additional information on the new biosketch requirements can be found at: <http://grants.nih.gov/grants/guide/notice-files/NOT-OD-15-024.html>.

**NOTE:** Anticipated/planned **INTEGRATED** QOL/PRO study applications should be annotated on the respective CTEP/NCORP *Trial Concept Submission Form* and must be submitted **within three (3) months** of PI notification by the respective CTEP/DCP PIO, that the concept was approved. Subsequent NCI prioritization and approval for funding will be decided by CTROC after evaluation of the study(s) by the respective NCI Steering Committee (SC).

1. The QOL/PRO study application has been discussed with DCP staff and it has been determined that the collection of data requires resources beyond the usual DCP Cancer Control credits.  
 YES          DCP staff member \_\_\_\_\_
2. State the symptom science/QOL/PRO hypothesis(es) and its scientific foundation. Specify the study endpoint(s).
3. Identify the QOL/PRO instrument(s) to be used to test each hypothesis, the basis for choosing each instrument, and the timing of the assessments.
4. For each instrument, document its validity, reliability, and responsiveness in the selected patient population. Specify the minimum important difference (MID) or metric for clinically-significant change. Applicants are encouraged to submit a symptom science/QOL/PRO Standard Operating Procedure (SOP) as an appendix, to support validation of the test/tool/instrument(s) being proposed.
5. For each instrument, identify whether it is INTEGRAL or INTEGRATED.
6. Describe any included *objective* correlates that enhance the patient-reported outcomes data (e.g. actigraphy, imaging, pulse ox, etc).
7. Explain how patient non-compliance, missing data and/or early death may impact the analysis.
8. How will visually-challenged, non-English speaking patients be accommodated when completing the instrument(s)?
9. Describe the procedures for data collection and data monitoring including the training of data collection personnel.

10. Provide turn-around-time for reporting instrument results to clinical PI (for INTEGRAL studies).
11. The Budget Justification should provide cost comparisons to justify the site(s) chosen to complete the assessment/test, where applicable. Justification should include potential cost-sharing approaches for the assessment/test (e.g., billing to third-party payers, partial funding from commercial partners, etc.), as well as cost comparison and justification for academic vs. commercial settings.

**Please complete and return the attached  
QOL/PRO STUDY EVALUATION TEMPLATE.  
Thank you.**

3/09,3/10,3/11,3/12,11/13,12/14,2/17