Effective Health Care

Progression-Free Survival
Nomination Summary Document

Results of Topic Selection Process & Next Steps

- Progression-free survival as a surrogate endpoint for functional outcomes or overall survival in evaluation of cancer treatments is not feasible for a comparative effectiveness review because it represents a method for evaluating interventions, rather than an actual healthcare intervention. However, it is an important topic and may be considered for a potential methods project within the Effective Health Care (EHC) Program.

Topic Description

Nominator: Organization

Nomination Summary: The nominator questions whether there is evidence related to use of image-based disease free progression as a substitute for functional outcomes/overall survival for any particular subgroup or for any particular malignancy. The nominator notes that their interests are broadly focused on outcomes but these include traditional measurements such as drug toxicities, quality of life (Q0l), and overall survival (OS).

Staff-Generated PICO
Population(s): Patients with cancer who are in clinical trials of cancer drugs that are being treated with a drug that improves progression-free survival (PFS)
Intervention(s): Measurement of PFS using imaging or other biomarker
Comparator(s): Measurements of quality of life (QoL), symptom relief, drug toxicities, or initiation of next line therapy
Outcome(s): The relationship between PFS and other measures of improved outcomes

Key Questions from Nominator:

1. What is the evidence for use of "diagnostic image" determined progression free survival (PFS) as compared to "symptom based" progression free survival or other outcome measures, as a surrogate for use in determining effectiveness of interventions in the treatment of malignancies?

Considerations

- The topic meets all EHC Program selection criteria. (For more information, see http://effectivehealthcare.ahrq.gov/index.cfm/submit-a-suggestion-for-research/how-are-research-topics-chosen/.)

- Overall survival has long been considered a highly useful, objective measurement on which to base clinical risk-benefit decision making in cancer research about the use of a new drug or new intended use of an established drug. However, using overall survival as the outcome measure in a clinical trial requires extended follow up of patients and may be affected by subsequent therapies that the patients receive. The use of alternative endpoints can allow for shorter clinical trials and limit the effects of outside factors on the results of the trial. A number of alternative endpoints have been identified to assist in the study of drugs, including the measurement of progression-free survival. However, it is unclear whether many of these endpoints represent a clinical improvement and benefit to patients. A methods project on this topic could examine the correlation of overall survival with alternative outcomes measures, including quality of life, based on the published literature.