

Summary

The percentage of elderly, over the age of 65, in the U.S. population will be changing dramatically over the next 20-30 years. The changing demographics over the next three decades will have significant consequences for Medicare and national policy. Given the economic impact of future health care expenditures by our growing elderly population, a concerted effort needs to be made to define high quality yet cost-effective medical therapy for older patients.

The purpose of this dissertation is to explore the use of both clinical trials and evidence-based decision models in performing cost-effectiveness analysis in elderly patients. For some diseases, such as cataract surgery, the majority of patients tend to be older. Therefore, previous studies focusing on younger patients do not exist. Important therapeutic and policy questions can only be addressed through a clinical trial. Other diseases, such as breast cancer, involve a wider age range of patients from early 40s to 90s. For such diseases, there is a literature of clinical trials on younger patients and the young elderly, 60 – 70. This previous literature can be used to develop decision analysis models to help define pertinent questions and areas for further research (i.e., clinical trials).

This dissertation is broken up into two main parts, demonstrating two different approaches to cost-effectiveness in an older population, a clinical trial and modeling from existing data. One part focuses on a randomized clinical trial on cataract surgery. The other part develops an evidence-based decision analysis model on the cost-effectiveness of treating older patients with early breast cancer.

The cataract surgery section has two sub-components: (a) a methodological section focusing on strategies to deal with question non-response among the older patients on the Health Utilities Index Mark 3, HUI3, questionnaire, and (b) a cost-effectiveness analysis based on a randomized clinical trial of older patients with cataracts comparing up-front surgery versus watchful waiting in patients who have relatively good visual functioning. HUI3 analysis demonstrated a significant percentage of missing data due to “Don’t Know” responses that could be handled using inspection/deduction from the pattern of completed responses. In the cost-effectiveness analysis, the use of a preoperative tool, Cataract Surgery Index, was shown to discriminate between those with high and low probability for improvement from cataract surgery, and those for whom surgery was cost-effective.

The breast cancer section focuses on an evidence-based decision analysis for older patients, >65, who have newly diagnosed early stage breast cancer. This analysis includes models taking into account longevity, aggregate comorbidity, frailty, and established preferences from quality of life literature. The uncertainty associated with treatment decision in older breast cancer patients could be mapped in this decision analysis framework. Whereas in younger estrogen positive breast cancer patients adjuvant chemotherapy was a dominant strategy, in older patients the dominant strategy was hormone therapy. In both 65 and 75 year old patients, there were scenarios for which combined hormone and chemotherapy could be considered cost-effective. Furthermore, sensitivity testing taking into account higher discount rates in older patients and different baseline quality of life states altered the cost-effectiveness of most adjuvant therapy strategies.

Many policy decisions will be made in the future pertaining to the provision of health among elderly patients. A broad set of approaches will be required to determine the cost-effectiveness of specific therapies in this population. These approaches will range from clinical trials to elaborate modeling using a combination of existing data and assumptions. The dissertation provides two examples using these approaches in performing cost-effectiveness analysis among the elderly.