

# Why Perspective Matters In Health Outcomes Research Analyses

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## ABSTRACT

*Health outcomes research typically uses cost-effectiveness or cost-utility analysis. These approaches take a narrow perspective of the individual effects, typically from the payer or the provider point-of-view. However, using these narrow perspectives misses macro-level, or societal level, benefits and costs that could significantly alter whether an intervention is considered beneficial or cost-effective. The societal perspective accounts for all the effects impacting patients, their families, the public, and government expenditures for a healthcare intervention. Such a perspective is vital for healthcare interventions for illnesses where morbidity and long absences from work are probable. A cost-benefit analysis would account for all the societal benefits and costs, allowing policy-makers to observe an outcomes analysis more closely reflective of the real impacts. This paper clearly presents why a societal perspective using cost-benefit analysis should be the preferred method of health outcomes research. An example of breast cancer interventions is used to illustrate this point.*

**Keywords:** Health Outcomes; Cost-Benefit Analysis; Cost-Effectiveness

## 1. INTRODUCTION

Cancer is the leading cause of death worldwide, accounting for approximately 13% of all deaths (WHO, 2013). Although early detection and treatment can help to increase survival rates, some unfortunate patients, of which many are poor (Freeman, 2004), develop metastatic cancer that has no cure. Palliative treatment is a main objective in this group of patients in order to prolong life and to reduce the side effects from interventions. The cost of treatment includes not only direct costs but also indirect costs. While the direct costs are obvious, including physician and medication costs, indirect costs are often not considered in economic evaluations such as in cost-effectiveness and cost-utility analysis studies of medical interventions like chemotherapy for cancer treatment. Although many studies have examined the economic burden and performed cost-effectiveness analyses of treatments for various diseases, most of the studies have been conducted from a payer perspective and reported only the direct costs. In fact, direct costs are the smallest portion of total costs per patient (Pallis et. al., 2010). As a result, indirect costs are important to include because of the large number of patients in the workforce and their economic impact (Pallis et. al., 2010). Indirect costs for a healthcare intervention are those that occur outside the medical sector, including lost productivity due to morbidity and mortality by patients. For instance, patients or caregivers might be unable to go to work or if they can work will not be able to work at full capacity. Indirect costs of cancer, including the absence from work and the impact on productivity, reduced productivity without absence from work, the influence of unemployment on production loss, and premature mortality and morbidity, are typically a substantial part of the macroeconomic impacts and are, as a result, relevant for health policy (Koopmanschap and Rutten, 1993). For example, if productivity is reduced due to sickness or death the employer will bear higher costs, such as having to hire new labor or paying overtime wages, which will ultimately be passed on to the consumer in the form of higher prices. Despite the importance of including these indirect costs in analyses of cancer treatment,

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only a few studies have reported them. Research on cancer patients has been centered on the direct medical care costs. However, given the nature of the healthcare system, the government funded by taxpayers (society) also bears costs, whether paying for healthcare services or reduced taxation revenue.

At the time, studies which included indirect costs only included loss of income to family members. Stommel, et al. (1993) were among the first to include the employment conditions of the family members of patients, the patient and caregivers used to care for the cancer patients. They estimated the costs based on the market value of the lost labor time. Additionally, out-of-pocket costs were included, covering all cancer care-related expenses that were not reimbursed to the patient by third parties. They found that, including indirect costs, the average cancer home care costs over a three month period were \$4,563, as compared to a cost of \$5,704 for a three month stay in a nursing home in Michigan.

Fortner, et al. (2003) built on these findings to examine the predictors of direct and indirect costs associated with pain in cancer patients. They surveyed three hundred seventy-three cancer outpatients about pain-related direct and indirect costs, such as hospitalizations, emergency room visits, transportation costs, and complementary pain management methods. They found that 76% of patients had at least one pain-related cost, for an average monthly direct cost of \$891 per month per patient. Direct medical cost due to pain was reported by 69% of the respondents who indicated total direct pain-related costs of \$825 per month per patient. Indirect costs of \$61 per month per patient for at least one pain-related expense were reported by 57% of the patients. Higher pain intensity, greater pain interference, and the presence of breakthrough pain were found to be predictors for higher direct and indirect medical expenses.

Chang, et al., (2004) estimated that cancer accounts for nearly \$61 billion in direct medical costs and \$15.5 billion for indirect morbidity costs. They found that policy makers responsible for developing annual estimates of cancer costs should incorporate a broad range of data sources to include the indirect costs of work absence and short-term disability. They found that indirect morbidity costs to employees with cancer averaged \$945 due to a monthly loss of two workdays and five short-term disability days.

For instance, Sorensen et al. (2012) estimated the indirect costs of metastatic breast cancer treatment in the US. They created an incidence-based cost-of-illness model to estimate the annual societal costs associated with treatment of metastatic breast cancer. Their model simulated costs of \$12.2 billion over a five year for disease management phases including active cancer treatment, palliative or supportive care, and end of life care. Total direct costs were estimated to be more than \$9.3 billion or \$75,415 per patient-year and indirect costs projected to be \$2.6 billion or \$21,153 per patient-year over 5 years.

The rest of paper is organized as it follows. Section 2 provides an overview of the standard health outcomes research approach to healthcare interventions. Section 3 examines how a societal perspective to healthcare analyses is a better approach to determining the outcomes of an intervention, and Section 4 concludes the paper with discussion and policy implications of a societal approach to health outcomes research.

## **2. STANDARD HEALTH OUTCOMES APPROACHES**

The previously discussed studies have shown the high costs associated with healthcare, especially for cancer treatment. In the US, to address this issue, new financing and healthcare policies have been developed, such as the 2010 Patient Protection and Affordable Care Act. As a result, economic evaluation is one of the most important aspects of developing healthcare policy and assessing any new intervention.

To guide policy makers on how to allocate limited healthcare resources in the most efficient and effective manner, many economic evaluation methods have been developed under the umbrella of health outcomes research. These evaluation methods can be used to measure the costs, benefits, and impacts of healthcare innovations and typically include cost-minimization analysis, cost-effectiveness analysis, and cost-utility analysis.

To date, economic evaluation and health outcomes studies have focused heavily on cost-effectiveness analysis and cost-utility analysis which generally use quality-adjusted life years (QALYs) as a measurement. However, QALYs are not a valuation measure, but a form of health benefit. Furthermore, a QALY is not connected

to any underlying economic theory. Cost-benefit analysis on the other hand is solidly connected to economic theory but it is rarely used in healthcare intervention evaluations because people are uncomfortable measuring healthcare interventions in monetary terms, the standard unit in cost-benefit analysis. The reason for this fear is that policy-makers and the public will focus too much on the dollar figure; that policy-makers might develop an arbitrary monetary cutoff point and the public overly concerned by healthcare methods that might be inexpensive.

Each method uses distinct measurements to demonstrate the benefits of healthcare interventions. Cost-minimization analysis assumes that all healthcare interventions have similar efficacy and only the costs are compared for different treatment options with the lowest cost alternative chosen. Cost-effectiveness analysis is an economic evaluation tool that has been widely used in healthcare. This method measures costs in monetary terms and benefits in natural units of effectiveness, such as life-year gained. Cost-utility analysis, which sometimes is considered a subtype of cost-effectiveness analysis, calculates all costs in monetary units and all benefits in terms of QALYs. Cost-benefit analysis measures all costs and benefits in monetary units.

Where economic evaluations are concerned, there are two types of comparisons of healthcare interventions: independent and mutually exclusive. For an independent intervention costs and outcomes are not affected by other interventions. For example, a patient might receive both a chest x-ray and a MRI in order to diagnose his or her condition. If interventions are independent, the average cost per effectiveness unit will be used to compare the interventions. For example, an average cost-effectiveness ratio is calculated by dividing the total cost of an intervention by the total outcomes or health effects produced, such as life-years gained. An average cost-utility ratio can also be used for independent interventions. Cost-utility ratios are calculated by dividing the total cost of an intervention by QALYs.

On the other hand, mutually exclusive interventions can be applied to the same population group but not to the same individual for the same condition (Stinnett and Paltiel, 1996). For example, two patients could have the same diagnosis but each would have different medication dosages or a different treatment. That is, the use of one intervention prevents the use of another. Mutually exclusive interventions are evaluated by examining how the costs and consequences of one intervention will be affected by other interventions. Furthermore, implementing other interventions is not possible. Mutually exclusive interventions use incremental cost effectiveness ratios calculated by dividing the difference in costs between the two interventions by the difference in health effects between the two interventions. As a result, incremental cost-effectiveness ratios start from the current level of benefits. Since cost-effectiveness analysis considers only health benefits it may underestimate the total benefits of health interventions that improve productivity and quality of life.

However, the objective of cost-effectiveness analysis is to maximize the effectiveness of an intervention based on budget constraints. The effectiveness of interventions are measured in life-years gained and QALYs gained for cost-effectiveness and cost-utility analysis respectively. Mutually exclusive options rank interventions from the highest to the lowest effectiveness. Then, programs that have a higher cost and lower effectiveness are ruled out as a dominated alternative, so they should not be implemented. The remaining interventions have their incremental cost-effectiveness ratios calculated. Using a fixed budget approach, a price per effectiveness unit, such as cost per life-year gained and QALY gained can be used as a decision rule. That is, the program that has the incremental cost-effectiveness ratio greater or equal to the price per QALY should be implemented. Therefore, this decision rule maximizes the healthcare budget, assuming constant returns to scale; the scale of a healthcare intervention can be reduced without changing the incremental cost-effective ratios. Under the assumption of constant returns to scale, only healthcare costs are included in the cost-effectiveness analysis. As the empirical data shows, QALY's do not include changes in income (Davidsson and Levin, 2008) or productivity effects (Jonsson, 2009).

Several studies have incorrectly used the healthcare budget maximization approach stating that it is a societal perspective. In fact, these papers have been criticized because, for the healthcare budget maximization approach, the theoretical foundation of cost-effectiveness obtained from welfare economics is unclear. Furthermore, this approach does not consider all future changes in healthcare costs and could lead to suboptimal decisions because only the prices faced by the budget holder will be considered, ignoring opportunity costs. Therefore, using a constant willingness-to-pay per an effective unit gained may be a more precise method because it will lead to cost-effectiveness analysis and cost-benefit analysis providing similar results.

As a result, health outcomes studies have focused primarily on cost-effectiveness analysis and cost-utility analysis. However, cost-effectiveness and cost-utility analysis studies have largely been conducted from a narrow perspective, typically from the payer (i.e. the patient) standpoint, resulting in a narrow cost consideration that does not reflect the true costs and benefits or consequences of health interventions. Health outcomes studies based on a societal perspective are vital for better healthcare and improved resource allocation.

This paper considers a societal perspective is necessary when all the costs and benefits of a healthcare intervention impact the society as a whole including patients, insurance agencies, employers, and the government. In other sectors of the economy, such as education, the economic evaluation of investments is done using a societal perspective. Consequently, since healthcare and education are competing for public financial resources it is better to evaluate healthcare interventions using cost-benefit analysis so the populace and policy-makers can better understand the analyses. Not performing health outcomes research from a societal perspective might result in a bias against investments through health care spending (Jonsson, 2009).

### **3. SOCIETAL PERSPECTIVE VS. PAYER OR PROVIDER PERSPECTIVES**

Perhaps the best evaluation tool from a societal perspective is cost-benefit analysis. Cost-benefit analysis measures all costs and benefits in monetary units and is part of applied welfare economics (which aims to define and value individual preferences that help to improve social welfare in order to maximize the usage of resources in an effective way). On the other hand, from an economic welfare standpoint, cost-effectiveness analysis conducted from a societal perspective would have to assess all the costs and health outcomes for the entire population. Furthermore, cost-effectiveness analysis that takes a societal perspective could lead to the neglect of some parties due to misallocation. Moreover, non-health benefits cannot be incorporated into cost-effectiveness analysis. Therefore, using cost-effectiveness analysis to measure health outcomes does not emphasize true societal objectives and values. In fact, if non-health benefits, such as the income of family members, from an intervention are not trivial, performing a cost-benefit analysis would be essential to assess the true benefits.

In the case of analyzing the outcomes of cancer interventions, most research has centered on cost-effectiveness or cost-utility analysis. Consider the case of treatment for breast cancer where both a nanotechnology developed medicine, nab-paclitaxel, and a standard medicine, cremophor or solvent-based paclitaxel, can be used. A number of research groups (Lazzaro et. al., 2013; Alba et. al., 2012; Maran et. al., 2012)<sup>2</sup> have examined these drug options using a cost-effectiveness analysis to evaluate the clinical and economic outcomes in different countries including the United States, Canada, the United Kingdom, France, Germany, Italy, Spain, and Portugal. However, these previous studies have considerable limitations due to the choice of using cost-effectiveness or cost-utility analysis. First, those studies have been conducted from payer and provider perspectives rather than from a societal point-of-view. Conducting health outcomes evaluations from such narrow perspectives could significantly undermine the true benefit of an intervention for society. Using cost-benefit analysis to determine the health outcomes of an intervention would substantially enhance the ability of decision-makers to make better healthcare policy. A cost-benefit analysis will incorporate all benefits, not just the limited benefits included in a cost-effectiveness analysis. Including societal surplus into a health outcomes analysis is vital because the entire benefit and cost of a new intervention can be seen. Such an analysis would include all the indirect costs and benefits to society that are not included in cost-effectiveness or cost-utility analyses. Typically, only direct healthcare and drug costs are included in cost-effectiveness or cost-utility analyses.

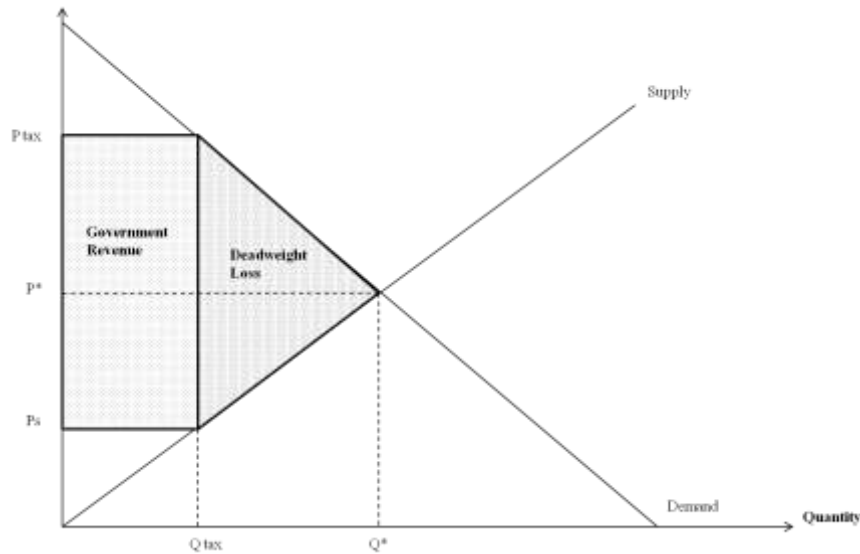
In the example provided earlier, according to cost-effectiveness or cost-utility analysis nab-paclitaxel is a cost-effective intervention. However, if cost-benefit analysis is used and a societal perspective included, nab-paclitaxel might not be a cost-effective strategy. Recently, the generic version of paclitaxel was delivered to the market and is 40 times less expensive than nab-paclitaxel. As a result, policy-makers could be led to think generic paclitaxel is the better intervention. However, previous studies did not include the indirect and opportunity costs, such as days lost of work.

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<sup>2</sup> These are just a few examples of a substantial amount of research published on the topic. We encourage the reader to follow his/her own curiosity and read that research.

Therefore, we recommend that indirect costs should be calculated from lost work productivity of patients due to missed days of work from sickness, impairment, premature death, as well as the burden of the informal caregiver in the form of lost leisure time, lost work hours due to absenteeism, and lost productivity due to missed days of work due to caring for their loved one.

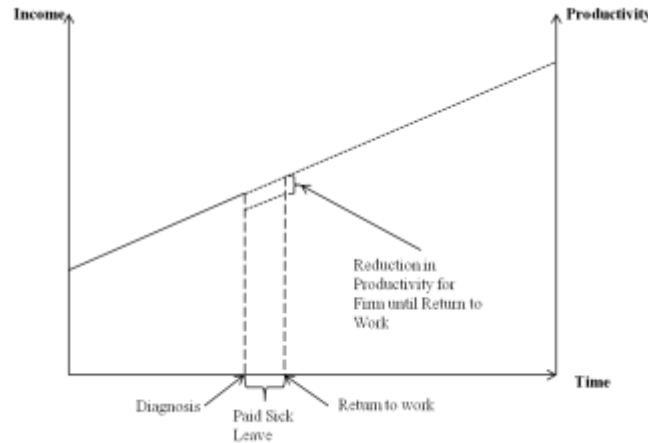
Furthermore, foregone income for the patient must also be considered. Chirikos et al. (2002) found that over a five year period breast cancer survivors in the United States suffered a larger reduction in annual market earnings and lower total household earnings than working control subjects. Consumers also suffer as a result of the high incidence of cancer. As incidences of disease, in this case cancer, increase, there will be a reduction in labor supply as those workers leave the workforce, temporarily in the case of those who survive, while getting treatment and permanently for those who die. There are over 1.6 million cases of cancer annually in the United States alone (American Cancer Society, 2012). As a result, if even half of those patients leave the workforce, at least temporarily, the reduction in the labor supply will create an upward pressure on wages. The increase in wages will cause inflation in the price of goods and services thus reducing consumer demand. Eventually, the reduction in consumer demand will cause profits for firms to decrease leading to a reduction in wages and labor demanded.



**Figure 1: Deadweight Loss from Taxation**

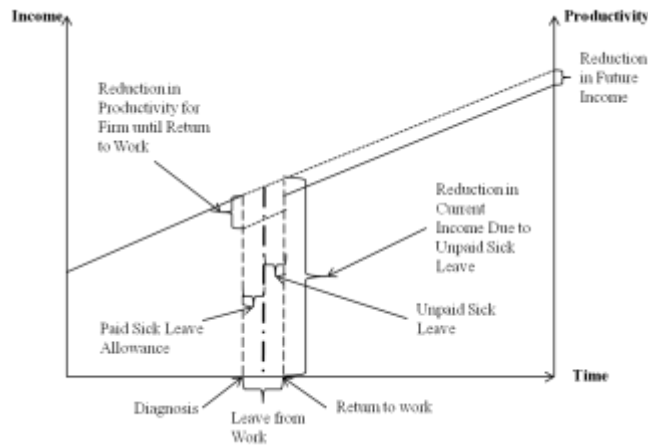
As shown in Figure 1, the public will also experience additional costs. The government will have to pay for or subsidize a percentage of the treatment for these cancer cases and need to generate additional revenue in the form of higher taxation to meet the costs of public services provided to patients. The tax will result in lower consumer demand from  $Q^*$  to  $Q_{tax}$  due to higher prices for goods and services, paying  $P_{tax}$  instead of  $P^*$ . The resulting deadweight loss is borne by society. As a result, the public pays higher prices while also experiencing the dissatisfaction of unmet demand.

Consider the following examples in Figures 2 through 5. These figures present a very simplistic model that assumes wages are equal to productivity and that this relationship is linear in time. Furthermore, any time missed from work might result in lost raises (depending on the length of time the patient missed from work) and the patient does not receive disability insurance unless the illness forces him or her to permanently not be able to work. However, while basic, these figures illustrate the effects that cost-benefit analysis captures that cost-effectiveness or cost-utility analyses do not. As illustrated, the societal impacts of lost productivity, foregone wage increases, lost income, and impact on family members and caregivers are significant and should be part of any healthcare intervention analysis. Since these benefits and costs are in monetary terms, using a cost-benefit analysis is practical and more efficient.



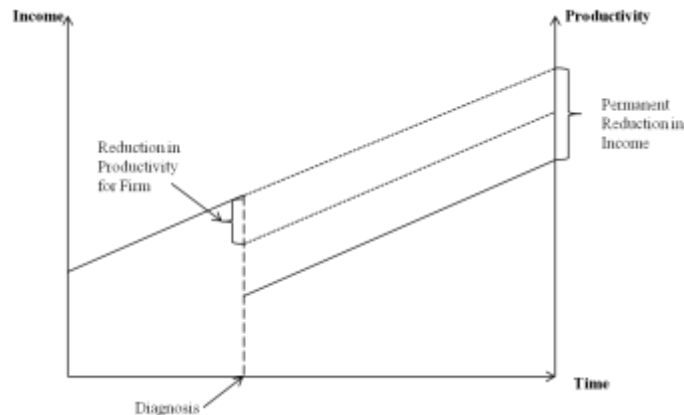
**Figure 2: Minimal Productivity Loss, Income Covered Entirely by Sick Leave**  
 Source: Access Economics (2007) with Authors' Adaptation

Figure 2 shows a best-case scenario for a cancer patient who is able to return to work before his or her sick leave expires. In this scenario, the worker does not suffer a loss in income and the firm loses productivity only during the time the worker is on leave. As a result, the patient does not forgo any future wage increases and the firm suffers minimal adverse effects.



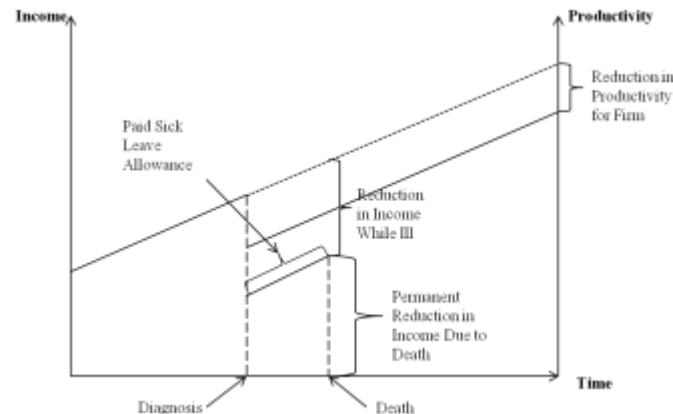
**Figure 3: Minimal Productivity Loss, Income Covered Partially by Sick Leave**  
 Source: Access Economics (2007) with Authors' Adaptation

The next scenario, illustrated in Figure 3, is not as positive for the worker. In this scenario, the illness is so prolonged that all his or her sick leave is used and the worker must take unpaid leave. In this case, the workers' income is not impacted while on paid sick leave. Once paid sick leave is used, their income will fall to zero until they are able to return to work, upon which time their income will be reinstated. However, given the time off on unpaid leave, the patient will have lost earnings increase opportunities and, as a result, their future income will be lower over time than it would have been otherwise. The firm, as in the previous scenario, loses productivity while the worker is on leave.



**Figure 4: Significant Productivity and Income Loss Due to Permanent Disability**  
 Source: Access Economics (2007) with Authors' Adaptation

The third scenario, Figure 4, shows a case where a worker is permanently disabled due to his or her cancer illness. In this scenario, the worker receives disability pay which is a percentage of his or her normal wages. As a result there will be a permanent reduction in income. In regards to the firm, since the patient is permanently removed from the labor force, there will be a lasting reduction in productivity. The firm, in response, will incur a cost by having to hire additional labor to compensate for the lost production. Additionally, the firm experiences a loss from the cost of the job search and training the new employee.



**Figure 5: Significant Productivity and Income Loss Due to Death**  
 Source: Access Economics (2007) with Authors' Adaptation

The final scenario, depicted in Figure 5, presents the case when the patient is diagnosed and dies from his or her cancer illness. In this case, the patient suffers a reduction in income while ill. Upon death, income goes to zero, adversely impacting his or her family financially. The firm also suffers negatively as they must hire a new worker to compensate for the lost production. The firm also incurs a cost of having to do a job search, hiring and training a new worker.

This simplistic model illustrates the effects that cost-benefit analysis captures while cost-effectiveness and cost-utility analysis do not. Furthermore, while just some of the societal impacts of cancer are shown, the example illustrates the significance of these effects and why the societal perspective must be a part of healthcare interventions, especially for diseases such as cancer where patients, family members, firms, and caregivers are affected.

#### 4. CONCLUSION

Health outcomes studies of healthcare interventions have predominantly used cost-effectiveness and cost-utility analysis. Sections 1 and 2 of this paper have shown how these analyses have serious limitations because they were conducted from a narrow perspective, such as from the payer (i.e. patient) or provider (i.e. hospital) point-of-view, and only considered the direct costs in their analysis. By restricting the point-of-view and neglecting to include indirect costs, the analysis is likely to be biased and could significantly undermine the true benefit of the interventions for society. Furthermore, since cost-effectiveness analysis can be considered a subset of cost-benefit analysis, the cost function for producing health effects is estimated regardless of who pays. As a result, cost-effectiveness analysis assumes that the willingness to pay per health effects is constant and homogeneous. However, the prices per unit of health effects are dependent on a variety of factors, such as age, that nullify constant willingness to pay and make cost-benefit analysis (Johannesson, 1995) the best choice for estimating health outcomes. While not perfect, in terms of measuring the societal effectiveness of a healthcare intervention, cost-benefit analysis is a better choice than cost-effectiveness or cost-utility analysis because cost-benefit analysis incorporates both health outcomes gained from individuals and the value gained to society. A cost-benefit analysis includes all the indirect costs, such as the productivity or transfer costs. Section 3 of this paper, using a simplistic model, showed just how significant these impacts are and why they must be part of any analysis of healthcare interventions.

As shown in the example in Section 3, the individual patient is the most adversely affected party; however, both family members and society also face costs of illness, particularly cancer. To best guide policy makers on how to allocate limited healthcare resources in the most efficient and effective manner, performing a cost-benefit analysis is essential because the outcomes are from a societal perspective. Given the changes that have occurred to healthcare systems and the budget pressures that exist for much of the governments around the world, a societal perspective is necessary to illustrate whether public investments in healthcare are beneficial or not. Such a perspective will inform the public and lead to better democratic decisions. That is, realizing societal surplus or total surplus is vital because the public will be able to understand the whole benefit and cost of an intervention.

This paper makes an important contribution to the existing literature because there is a clear outline why healthcare interventions, particularly those for cancer where technological advancements are resulting in new treatment options on a regular basis, should be analyzed using a societal perspective rather than the traditional health outcomes approaches of a payer or provider perspective. Only when a societal approach is used for healthcare interventions, especially when morbidity or prolonged periods of illness prevent the patient from working, the true measure of a healthcare intervention will be shown. Then, and only then, can policy-makers appropriately make a proper decision on financing healthcare.

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