

# **Estimating Medical Costs for Regulatory Benefit-Cost Analysis: Conceptual Framework and Best Practices**

**June 2017 Final Report**

**Prepared for:**

Amber Jessup, Senior Economist  
Office of Science and Data Policy  
Office of the Assistant Secretary for Planning and Evaluation  
U.S. Department of Health and Human Services

**Prepared by:**

Lisa A. Robinson, Harvard University (Center for Risk Analysis and Center for Health Decision Science)  
David B. Rein, NORC at the University of Chicago (Public Health Analytics Program)  
James K. Hammitt, Harvard University (Center for Risk Analysis and Center for Health Decision Science)

**With assistance from:**

John S. Wittenborn, NORC at the University of Chicago (Public Health Analytics Program)  
Ruth M. Wycle, NORC at the University of Chicago (Public Health Department)

**Under subcontract to:**

Jennifer R. Baxter, Industrial Economics, Incorporated  
Industrial Economics Incorporated  
GSA Contract Number: GS-10F-0061N

## **ACKNOWLEDGEMENTS**

This report was developed for Amber Jessup in the Office of the Assistant Secretary for Planning and Evaluation of the U.S. Department of Health and Human Services (HHS), under Industrial Economics, Incorporated's (IEc's) General Services Administration Contract Number GS-10F-0061N. It was prepared by Lisa A. Robinson (Harvard University, Center for Risk Analysis and Center for Health Decision Science), David Rein (NORC at the University of Chicago), and James K. Hammitt (Harvard University, Center for Risk Analysis and Center for Health Decision Science). John S. Wittenborn and Ruth M. Wygle (NORC at the University of Chicago) provided substantial advice and assistance.

We thank Amber Jessup as well as Scott Grosse (Centers for Disease Control and Prevention (CDC), HHS) and Clark Nardinelli and Bradley Brown (Food and Drug Administration, HHS) for their helpful comments on earlier drafts. Scott Grosse also shared his 2016 slides, "Cost of Illness Studies: An Overview," used in training CDC Fellows, which were very helpful in identifying related resources as well as key issues. In addition, we thank the peer reviewers for their useful comments on the July 2016 draft report: Paul G. Barnett (VA Palo Alto Health Care System), Anirban Basu (University of Washington), Joseph Lipscomb (Emory University), and Louise Russell (Rutgers University).

## TABLE OF CONTENTS

Acknowledgements.....	2
Table of Contents.....	3
Executive Summary.....	4
1.0 Introduction and Background .....	6
2.0 Conceptual Framework.....	8
2.1 Welfare Economic Principles .....	8
2.2 Distinguishing “Benefits” and “Costs” .....	10
3.0 General Approach .....	12
3.1 Basic Steps.....	12
3.2 Evaluation Criteria.....	15
4.0 Summary and Conclusions .....	24
References .....	25

## EXECUTIVE SUMMARY

The U.S. Department of Health and Human Services (HHS) is required to assess the benefits and costs of its major regulations prior to promulgation. To support these assessments, in 2016 HHS issued its *Guidelines for Regulatory Impact Analyses*, developed under the leadership of its Office of the Assistant Secretary for Planning and Evaluation and its Department-wide Analytics Team.<sup>1</sup> When developing these *Guidelines*, the Analytics Team recognized that HHS analysts needed more detailed guidance on estimating the medical costs of illness. This report supports the process of creating such guidance, building on the general framework described in the HHS *Guidelines*.

Numerous databases and studies provide estimates of these costs, and different approaches can lead to noticeably different estimates. However, no established set of recommended best practices exists for developing estimates specifically for use in regulatory benefit-cost analysis. Such analysis is required for individual regulations expected to lead to benefits, costs, or transfers of \$100 million or more in any given year, as well as for most other significant regulations. These regulations rarely focus on the treatment of particular conditions; rather, they aim to increase food, drug, or medical device safety or alter Federal requirements for certain types of health care facilities. Medical costs may be incurred as a result of regulatory implementation, or may be averted due to the reductions in morbidity and mortality risks that result.

Typically, regulatory analysts lack the time and resources needed to conduct new primary research, and rely on available studies and databases to estimate medical costs. Related steps include the following.

- **Step 1: Clearly define the condition(s)** affected by the regulation, including the severity and duration and the characteristics of the affected population, as well as the types of medical costs likely to be affected.
- **Step 2: Conduct a criteria-driven literature review**, which includes a comprehensive search for relevant studies and evaluation of these studies against established criteria.
- **Step 3: Identify research gaps and options for addressing them.** The results of the literature review may be directly applicable to the regulation or it may be possible to adjust the available estimates to be more consistent with the regulation's effects. At times, it may make sense to supplement the available research with estimates from national databases, such as the Medical Expenditure Panel Survey (MEPS) or Healthcare Cost and Utilization Project (HCUP). The choice among these options depends on the importance of the estimates to the analytic conclusions, as well as the data, time, and resources available and the results of the literature review.

---

<sup>1</sup> The HHS 2016 *Guidelines* are available at: <https://aspe.hhs.gov/pdf-report/guidelines-regulatory-impact-analysis>; they are accompanied by a primer that is available at: <https://aspe.hhs.gov/pdf-report/guidelines-regulatory-impact-analysis-primer>.

- **Step 4: Implement selected approach and address uncertainty.** The final step involves implementing the selected approach, and assessing uncertainty both qualitatively and quantitatively.

Perhaps the most important component of this approach is the criteria used to evaluate the quality and applicability of the research under Step 2. Exhibit ES-1 summarizes the evaluation criteria discussed in more detail in this report.

#### **Exhibit ES-1: Evaluation Criteria**

##### **General Criteria**

1. Addresses the U.S. population.
2. Includes needed cost elements.
3. Publicly-available and well-documented.

##### **Quality Criteria**

4. Independently reviewed.
5. Estimates social opportunity costs.
6. Uses appropriate analytic methods.

##### **Applicability Criteria**

7. Relies on recent data.
8. Addresses individuals with similar characteristics.
9. Addresses similar health conditions.

Typically, no individual study will meet all of the criteria, and there is not a clear dividing line for identifying whether a study does, or does not, meet many of them. For example, it should be straightforward to determine whether a study addresses a U.S. population, but it may be more difficult to determine whether the individuals studied are sufficiently similar to the population affected by the regulation. Deciding whether to apply a study in a regulatory analysis thus requires judgment on the part of the analyst; analysts should describe the extent to which selected studies meet these criteria to aid reviewers in understanding the basis for their choices as well as the advantages and limitations of the approach. Clear discussion and assessment of the implications of related uncertainties is necessary.

## 1.0 INTRODUCTION AND BACKGROUND

Under Executive Orders 12866 and 13563 (Clinton 1993, Obama 2011), the U.S. Department of Health and Human Services (HHS) is required to assess the benefits and costs of its major regulations before issuing them.<sup>2</sup> Such analysis is required for individual regulations expected to impose benefits, costs, or transfers of \$100 million or more in any given year, as well as for most other significant regulatory actions. These regulations may aim to increase food, drug, or medical device safety or alter Medicare requirements for certain types of facilities. While the number of major regulations is relatively small, their impact is substantial. For example, over the past 10 fiscal years, the U.S. Office of Management and Budget (OMB) reviewed 17 major HHS regulations for which both benefits and costs were estimated (OMB 2016, Table 1-1). In total, the annual benefits of these 17 regulations were estimated as \$5.2 billion to \$22.6 billion; the annual costs were estimated as \$1.6 billion to \$5.7 billion (2014 dollars, ranges reflect uncertainty in the estimates).<sup>3</sup>

To support these assessments, HHS recently finalized its *Guidelines for Regulatory Impact Analyses* (HHS 2016), under the leadership of its Office of the Assistant Secretary for Planning and Evaluation (ASPE) and its Department-wide Analytics Team. When developing these *Guidelines*, the Analytics Team recognized that HHS analysts needed more detailed guidance on estimating the medical costs of illness. Numerous databases and studies provide estimates of these costs, but are designed for a variety of purposes -- such as budgeting, reimbursement decisions, and priority-setting as well as policy analysis (see, for example, Yabroff et al. 2009a). Comparison of results across studies suggests that different approaches can lead to noticeably different estimates (e.g., Bloom et al. 2001, Honeycutt et al. 2009, Onukwugha et al. 2016). However, no established set of recommended best practices exists for developing medical cost estimates for regulatory benefit-cost analysis, and the methods used for other purposes are not necessarily appropriate for this context. Most work on medical costing has been completed to support cost-effectiveness analyses of specific interventions (such as the use of statins to reduce cardiovascular disease risk) or cost-of-illness studies for particular conditions or groups of conditions (such as all cases of cardiovascular disease nationally), rather than the more broadly-focused major national regulations that are the subject of this report.

---

<sup>2</sup> The majority of the work on this report was completed prior to January 2017. However, its findings will be useful for those conducting analyses to comply with Executive Order 13371, "Reducing Regulation and Controlling Regulatory Costs" which was issued on January 30, 2017. Related guidance is provided in OMB (2017).

<sup>3</sup> More information on these regulations and the supporting analyses is provided in annual OMB reports to Congress. In fiscal year 2015 (October 1, 2014 to September 30, 2015), OMB reviewed three major HHS regulations for which both costs and benefits were estimated: one addressing manufacturing practices for animal feed, one addressing nutrition labeling in restaurants and other retail food establishments, and one addressing the Medicare Shared Savings program (OMB 2016, Table 1-1(a)). In addition, OMB reviewed several major HHS regulations for which less comprehensive analyses were provided, including three food safety regulations for which only costs were estimated, one regulation related to the coverage of preventive services for which neither costs nor benefits were estimated, and 11 regulations for which transfer payments were estimated (OMB 2016, Tables 1-2 through 1-7). These transfers primarily relate to the effects of changes in the Medicare and Medicaid programs on the Federal budget.

In this report, we initiate the process of developing best practice guidance for estimating medical costs in regulatory benefit-cost analysis, building on the general framework described in the HHS *Guidelines*.<sup>4</sup> We focus on developing a sound and feasible approach for estimating per person medical costs for the types of conditions and patients likely to be affected by major HHS regulations. We address direct medical costs, including those related to identifying, treating, and managing a health condition. These costs include the consumption of goods and services associated with inpatient care (such as hospitalization) and outpatient care (such as office visits and rehabilitation services), as well as testing, medications, devices, equipment, and associated overhead charges.<sup>5</sup>

At times, a regulation will impose costs on health care organizations that are not directly related to treating patients with a particular condition, such as those associated with requiring hospitals to purchase equipment, provide training, or submit reports. Valuation of these other impacts are covered by the HHS *Guidelines*, which discuss methods for estimating capital, administrative, and operating and maintenance costs associated with regulatory implementation as well as for estimating the value of health risk reductions and other regulatory benefits. The HHS *Guidelines* also discuss the valuation of changes in time use, which we explore further in a companion report (Baxter et al. 2017).

This report explicitly recognizes the constraints under which regulatory analyses are conducted as well as the difficulties associated with estimating medical costs. Agency analysts generally lack the time and resources needed to conduct new primary research, instead often relying on existing databases and studies. Thus our goal is to help analysts understand the advantages and limitations of the available data and research and to flag key issues for their attention, so as to encourage high quality analyses as well as appropriate characterization and assessment of uncertainty.

In addition, as indicated by the discussion that follows, estimating medical costs raises several difficult and at times contentious issues that cannot be fully resolved through a project of this size and scope. These issues are reflected in the diversity of methods currently used as well as in the work of several expert groups, such as the larger and more comprehensive efforts of the Second Panel on Cost-Effectiveness in Health and Medicine and the International Society for Pharmacoeconomics and Outcomes Research (ISPOR).<sup>6</sup> These and other efforts provide more insights into related issues and will affect the conduct of future studies, potentially increasing the availability of estimates appropriate for application in regulatory benefit-cost analyses as well as in other settings. This report is intended to introduce analysts to related issues and to provide a general framework for them to follow; analysts

---

<sup>4</sup> For convenience, we use the term “costs” throughout this report to refer to increases or decreases in medical costs, regardless of whether they are carried on the benefit or cost side of the ledger in the regulatory analysis. The categorization of impacts as benefits or costs is discussed in the HHS *Guidelines*.

<sup>5</sup> In this report, we do not discuss how to value the costs of the time patients spend seeking medical care, although such time may be included as one of the costs of health production (see, for example, Russell 2009, Neumann et al. 2016).

<sup>6</sup> The Second Panel released its report (Sanders et al. 2016, Neumann et al. 2016) after the draft of this report was sent out for peer review. Two of the four peer reviewers of that draft were Panel members (Anirban Basu and Louise Russell), and their recommendations have been incorporated into this version of our report. ISPOR best practice reports are available at: [http://www.ispor.org/workpaper/practices\\_index.asp](http://www.ispor.org/workpaper/practices_index.asp).

should review the references it cites as well as other sources to develop a more detailed understanding of these issues and of the advantages and limitations of the available data.

Below, we first provide a brief overview of the conceptual framework for regulatory benefit-cost analyses and its relationship to the estimation of medical costs, comparing and contrasting key issues that arise in this context with those that are relevant when conducting cost-effectiveness analyses or cost-of-illness studies. We then provide a step-by-step approach for developing estimates of medical costs that can be feasibly implemented given the data sources and research now available.

The HHS *Guidelines* address many other analytic issues that are not discussed in detail in this report. One is the role of organizational learning. Care must be taken to distinguish between costs of new and established technologies or approaches: a regulation that leads to the use of new practices may have higher costs in the near-term than in the longer term, due to the effects of learning and other factors. Another is adjustment for inflation and time preferences. The *Guidelines* indicate that estimates must be reported in constant (real) dollars based on price levels in a designated year, undiscounted and discounted at both 3 and 7 percent real annual rates. A third issue is the treatment of uncertainty and non-quantified effects. The *Guidelines* discuss both quantitative and qualitative approaches for addressing these issues, including the use of sensitivity analysis and probabilistic methods.

## **2.0 CONCEPTUAL FRAMEWORK**

In this section, we first briefly review the conceptual framework for regulatory benefit-cost analysis as described in the HHS *Guidelines* and elsewhere. We then discuss issues related to how costs and benefits are defined in different forms of economic evaluation. Medical costs are more often estimated in health-related cost-effectiveness analyses and in cost-of-illness studies than in regulatory benefit-cost analyses. As a result, most of the literature on best practices (cited throughout this report) focuses on these other types of analyses, and must be adapted to fit the regulatory benefit-cost analysis context.

Any type of economic evaluation can be conducted from a variety of perspectives, including that of an individual health care provider, government agency, or other organization, or of a particular community, region, or country. As discussed in more detail in the HHS *Guidelines*, regulatory benefit-cost analysis is always conducted from a national perspective. Thus in the discussion that follows, we focus on principles that are applicable in analyses that address society-wide impacts.

### **2.1 Welfare Economic Principles**

Three key principles from welfare economics deserve particular emphasis in considering how to appropriately estimate medical costs in regulatory benefit-cost analysis. First, within this framework, opportunity costs are the appropriate measure of value. This approach explicitly recognizes that using resources (such as labor or raw materials) for one purpose means they are not available for other uses. Thus the value of a resource is determined by its most productive or beneficial use.

Second, this framework generally assumes that each individual is the best judge of his or her own welfare (“consumer sovereignty”), which means that values should be based on the preferences of the



affected individuals. If an individual chooses to buy a good or service, presumably he or she values the good or service more than the other goods or services he or she could have used that money to buy. This means an individual's willingness to exchange money for different goods and services can be used to estimate their value. For an individual as well as a society, income (or more accurately, wealth) represents the total amount of resources that can be allocated across various goods and services, taking into account the ability to borrow or save so as to spread consumption over time. Thus values should ideally be estimated from an individual's perspective, then aggregated to determine the value of the good or service to society.

Third, this framework requires distinguishing between real resource costs and transfers. Transfers are monetary payments between individuals or groups that have little impact on the total resources available to society, such as taxes, fees, and surcharges. Because they are a benefit to recipients and a cost to payers, transfers are often ignored in calculating net benefits, and only considered when assessing the distribution of benefits and costs across different entities and individuals. However, transfers may change behavior in ways that affect individual and social welfare; the effects of such behavioral changes should be included in the benefit-cost analysis if significant.

All three principles are very challenging to implement when estimating medical costs. In competitive markets, prices generally provide reasonable estimates of opportunity costs. However, health care markets are significantly distorted by taxes, government regulation (including insurance mandates), disproportionate market power, information gaps and asymmetries, and other issues, which means that market prices may diverge significantly from opportunity costs. In addition, prices are not easily discernable due to the bundling of services and cross-subsidization. In health care, posted or nominal prices often differ substantially from actual payments and from the marginal costs of production.

Examples of the role of market power include the effects of monopoly and monopsony behavior. While monopoly rents (e.g., for new drugs) are a transfer from buyers to the monopolist, monopoly behavior can induce a welfare loss by restricting the supply of the product compared with the competitive equilibrium. In health care, the availability of insurance encourages consumers to buy products even when the market price exceeds the value to them, and hence products may be overused even at monopoly prices. Monopsony power (e.g., by managed care providers) can also restrict supply compared with a competitive equilibrium, but again the availability of insurance may encourage patients to consume more treatment than they would if they were required to directly pay its full cost.

Ideally, the values used would reflect the marginal opportunity costs associated with the regulation's effects over the time period covered by the analysis. However, generally only average values for previous years are available and these values may not reflect actual transaction costs. These limitations need to be considered when developing estimates for use in regulatory analysis. We return to these issues in Section 3.0, where we discuss the consistency of various sources of cost estimates with this framework, including the concept of opportunity costs.

## 2.2 Distinguishing “Benefits” and “Costs”

Much of the work on appropriately estimating medical costs has been conducted within the framework of health-related cost-effectiveness analyses or cost-of-illness studies, as reflected in the sources cited throughout this report. Although the framework for regulatory benefit-cost analyses is similar, these contexts differ in some respects and the terminology used is not entirely consistent.

In regulatory benefit-cost analysis, impacts are valued in monetary terms, and the summary measure is estimated net benefits (benefits minus costs). In health-related cost-effectiveness analyses, costs are compared to a non-monetary outcome measure, typically quality-adjusted life years (QALYs). The summary measure is the estimated incremental cost-effectiveness ratio (costs divided by the effect measure; i.e., by the associated change in QALYs).<sup>7</sup> In cost-of-illness studies, researchers focus solely on costs without comparison to changes in health or other outcomes. Typically, in these studies researchers distinguish between the “direct” costs of illness (i.e., expenditures on medical treatment) and the “indirect” costs of illness, particularly the effects on the patient and at times caregiver productivity. The summary measure in this case is an estimate of the total direct and indirect costs attributable to the illness.

These distinctions affect the categorization of impacts as costs or benefits. In cost-effectiveness analysis, the use of a ratio as a summary measure means that what is counted as a cost and what is counted as a benefit must be consistent across analyses for comparability. As a result, substantial effort has been devoted to clarifying which impacts should be categorized as costs and which impacts are included in the effect measure. In particular, there have been significant debates over whether productivity losses are included in QALY estimates, and hence whether including these losses in the cost measure leads to double-counting (see Neumann et al. 2016 for more discussion). In cost-of-illness studies, while there is some debate about what costs should be included (see, for example, Yabroff et al. 2009a), the distinction between costs and benefits is less of an issue since benefits are not estimated.

In benefit-cost analysis, the use of net benefits as the summary measure means that results can be compared across analyses regardless of which impacts are categorized as costs and which are categorized as benefits, as long as the sign (positive or negative) is correct. Thus the categorization of outcomes as benefits or costs may differ across analyses. However, the HHS *Guidelines* suggest that analysts follow a consistent approach to categorization for clear communication and ease of comparison.

Specifically, the *Guidelines*:

- define *benefits* as the value of the outcomes of a regulation or other policy, such as reductions in mortality or morbidity risks, including any countervailing risks; and,

---

<sup>7</sup> At times, WTP estimates are used to establish cost-effectiveness thresholds, to distinguish between those interventions that may, and may not, be worth pursuing. See, for example, Hirth et al. (2000) and Robinson et al. (2017) for more discussion.

- define *costs* as the value of the inputs required to implement a regulation or other policy, including the labor, capital, and materials, as well as any offsetting savings.

In other words, regulatory impacts are categorized following a production framework, where inputs (implementation costs) are used to create outputs (improved health and other benefits). More information on the effects included in each category and the approach to valuation is provided in the *HHS Guidelines*.

Under the *Guidelines*, the specific types of medical costs (or savings) to be included in the analysis depend on whether they are related to the benefits or the costs of the regulations. The *Guidelines* indicate that benefit values should be based on estimates of individual willingness to pay (WTP) or on monetized QALYs as a proxy when suitable WTP estimates are not available.<sup>8</sup> Presumably an individual's WTP encompasses both the pecuniary effects of the risk change (including out-of-pocket medical expenses and future earnings) and the non-pecuniary effects (such as pain and suffering and the joys of life more generally). This means that adding out-of-pocket expenses to WTP estimates could result in double-counting. However, individuals may not take into account costs paid by third parties in estimating their WTP, such as medical costs paid by government programs or private insurance.<sup>9</sup> Hence such payments may be added to WTP estimates when appropriate to more fully reflect the effects of the policy.<sup>10</sup>

The *Guidelines* indicate that the cost analysis will usually focus on the direct costs of regulatory compliance over time, including the labor, material, and capital used to meet the requirements. These costs are typically valued using market prices, with some exceptions when markets are significantly distorted.<sup>11</sup> Thus the cost analysis may include changes in per patient medical costs in those cases where the regulation requires alterations in medical practices for a particular condition or set of conditions.

For example, many of the major HHS regulations for which benefit-cost analysis is required address food safety. The costs of these regulations are likely to include those associated with protective measures undertaken by manufacturers and retail establishments, such as changes in production processes,

---

<sup>8</sup> The application of this approach, and its advantages and limitations, are described in more detail in the *HHS Guidelines*.

<sup>9</sup> These third party costs also include time losses associated with caregiving provided outside of the health care system (i.e., not covered by insurance); for example, by friends and family. As noted earlier, the valuation of productivity losses and other changes in time use are being addressed in a companion project, and are not discussed in this report.

<sup>10</sup> The specific costs to be included or excluded will depend on how they are addressed in the specific WTP studies used. For example, when stated preference surveys are used to estimate WTP, the costs that can be added to the resulting estimates will depend on whether the researchers instruct respondents to ignore or include out-of-pocket costs (as well as on any evidence that indicates whether respondents adhered to these instructions). If the relevant WTP studies do not explicitly include costs covered by third parties, costs such as those paid by health insurance may be added to WTP-based benefit values. If the underlying valuation studies explicitly exclude out-of-pocket expenses and lost production (or lost earnings), then these costs can also be added to the WTP estimates.

<sup>11</sup> Occasionally, regulatory compliance may noticeably affect prices, in which case the market response (in terms of changes in supply and demand) should also be taken into account (for example, by using partial equilibrium modeling).

employee training, inspections, and reporting. The benefits are likely to include reduced mortality and morbidity risks associated with foodborne illness. In such cases, there is no need to estimate medical costs as part of the cost analysis; regulatory implementation does not directly address the medical treatment of particular health conditions. However, such costs may be estimated as part of the benefits assessment. An individual's WTP to reduce the risk of foodborne illness is not likely to include the avoidance of costs covered by insurance, such as any needed hospitalization.<sup>12</sup> Hence estimates of such costs may be added to the WTP estimates to determine the total value of the risk reductions.

For some regulations, the particular medical costs or savings that should be counted will be uncertain, and analysts will need to document this uncertainty. Medical costs generally should be presented as a separate line item in the calculations so their treatment is clear. The estimation of these costs should follow the approach described below, regardless of whether they are included in the cost or benefit component of the analysis.

### 3.0 GENERAL APPROACH

The general approach for estimating medical costs is similar to the approach used to value other regulatory impacts, as described in the HHS *Guidelines*. Below, we first introduce the approach, then discuss the criteria that should be considered when evaluating the quality and the applicability of the available medical cost estimates.

#### 3.1 Basic Steps

The detailed implementation of the approach for estimating medical costs will depend at least in part on the importance of the medical cost estimates to the analytic conclusions and on the data, time, and resources available. As discussed in the HHS *Guidelines*, screening analysis should be used to provide preliminary information on the possible impact of the estimates and to inform decisions about future work.<sup>13</sup> Health economists who focus on the conditions of interest, such as the technical experts at the Centers for Disease Control and Prevention, can provide useful assistance.

The approach for estimating medical costs typically includes four basic steps.

- **Step 1: Clearly define the condition** or conditions affected by the regulation, including the severity and duration and the characteristics of the affected population, as well as the types of medical costs likely to be affected over time. As discussed in the HHS *Guidelines*, regulatory benefit-cost analysis involves comparing the world without the regulation (the baseline) to the world with the regulation. This means that, when determining what medical costs should be included in the analysis, analysts

---

<sup>12</sup> Some proportion of hospitalization and other insured medical costs is likely to be covered by the patient, due to deductibles and copayments. As noted in Section 3.0, related uncertainties should be addressed in presenting the analytic results.

<sup>13</sup> Screening analysis typically uses easily accessible data and simple assumptions to provide preliminary information on the possible direction and magnitude of regulatory impacts. For example, high-end values can be used to determine whether particular outcomes are likely to be significant even under extreme assumptions, and hence to identify those outcomes for which additional data collection efforts are most needed.

should identify the conditions whose incidence is likely to be affected by the regulation, estimate the change in incidence of each condition resulting from the regulation, and estimate the associated incremental medical costs, including sequelae or other effects on co-morbidity.<sup>14</sup>

Regulatory analyses often address impacts (e.g., cases of illness averted per year) over a 10 to 20 year period. However, for each case averted, the estimates should reflect the net present value of averted lifetime medical costs regardless of whether the costs accrue more than 10 or 20 years in the future.

In many cases, the most feasible and appropriate comparison for estimating these incremental costs will be between an average individual with the condition and a similar individual without the condition, starting at the same year of age and continuing over their remaining life expectancies. Under the subsequent steps, the characteristics of the conditions affected by the regulation then can be compared to the characteristics of those addressed by the available research, to evaluate the extent to which the effects of the regulation are similar to the effects studied.

- **Step 2: Conduct a criteria-driven literature review**, which includes a comprehensive search for relevant studies and evaluation of these studies. The search should include the peer-reviewed literature as well as reports and datasets developed by government agencies and other reputable groups.<sup>15</sup> Typically, bibliographic databases such as PubMed, the ISI Web of Science, and Google Scholar can be very useful. To locate additional references, analysts will often find it helpful to use the “cited by” feature in these databases in addition to running keyword searches, and should also check the citations in each study. Because these searches will not necessarily identify work that is in process or that was published by sources other than academic journals (such as government reports), analysts should also search the websites of relevant governmental and non-governmental organizations, and contact leading researchers to determine whether additional work is available.

The studies identified during this search should be evaluated against well-defined criteria for quality and applicability, as discussed in Section 3.2.<sup>16</sup> In general, “quality” refers to the expected reliability and validity of the data sources and methods used; “applicability” refers to the similarities of the conditions and populations studied to those affected by the regulation. For example, the available studies may address cases that are more or less severe, may include treatments that are now outdated, or may reflect recovery rates that differ from those experienced by current patients. They also may reflect local costs that are not representative of costs incurred nationally, or may focus on age groups that differ from those affected by the regulation. Often, they will cover average costs per case over a limited time period, rather than marginal lifetime costs per case. Substantial professional judgment is needed in weighing the advantages and limitations of the available

---

<sup>14</sup> At times, the regulation may affect the joint probability of occurrence for more than one condition.

<sup>15</sup> Lund et al. (2009) provides a useful inventory of the then-available databases.

<sup>16</sup> Some criteria, such as whether the study was conducted in the U.S., can be implemented as part of the initial search rather than as part of the subsequent evaluation.

research, given that no data source is likely to fully satisfy all of the evaluation criteria. These judgments must be clearly documented.

- **Step 3: Identify research gaps and options for addressing them.** The results of the literature review may be directly applicable to the regulation or may need to be adjusted to be more consistent with the regulation's effects. For example, a high quality study may be available that addresses both more and less severe cases, and analysts may be able to separate out the costs for the less severe cases if needed. For some conditions, it may be more sensible to extract data directly from available databases (such as MEPS or HCUP) rather than rely on a previously completed study. Bounding analysis may be useful when estimates vary significantly, or when data or other constraints limit the extent to which the available research can be adjusted to better fit the regulatory context. The choice among these options depends on the importance of the estimates to the analytic conclusions, as well as the data, time, and resources available and the results of the literature review. It is often useful to compare estimates from multiple sources to provide insights into related uncertainties.

This step often involves the use of research synthesis methods to combine the results of different studies and address inconsistencies in the findings, including systematic review, meta-analysis, and expert elicitation. The quality of the combined results depends on both the approach to synthesis and the approach used in the underlying studies, including the extent to which they meet the criteria discussed in Section 3.2.<sup>17</sup>

- **Step 4: Implement selected approach and address uncertainty.** The final step involves implementing the selected approach, and assessing uncertainty both qualitatively and quantitatively. Analysts should consult the HHS *Guidelines* for more information on addressing uncertainty, including the impacts of non-quantified effects, as well as on reporting the results.

The process described above is similar to the systematic review process frequently used in health care, described in the Institute of Medicine's (IOM's) 2011 expert panel report and elsewhere.<sup>18</sup> In that report, the authors review, update, expand, and refine standards previously developed by other organizations for comparative effectiveness research. They recommend 21 standards and 82 performance elements spanning four major steps: initiating the process, locating and assessing studies, synthesizing the evidence, and reporting the approach and results. The authors note, however, that systematic reviews are unlikely to fully adhere to these recommendations, which require substantial time and expense to implement. Analysts will need to review the recommendations and consider which are most appropriate and useful in a particular context.

The IOM (2011) and other frameworks for systematic review in health care often target a narrowly-defined intervention (e.g., the impact of a specific medication on a particular health condition), where

---

<sup>17</sup> See Robinson and Hammitt (2015) for an overview of these methods and their application to policy analysis as well as references to other useful resources.

<sup>18</sup> This framework is also very similar to the benefit transfer framework used to estimate the value of nonmarket outcomes, such as mortality and morbidity risk reductions, which is described in the HHS *Guidelines*.

medical costs often represent a substantial proportion of the impacts. Regulatory analysts are generally interested in broader categories (e.g., all costs associated with an average case of heart disease) and medical costs are likely to be a relatively small component of the overall regulatory impacts. Thus while we suggest that regulatory analysts examine the IOM and other systematic review frameworks to gain useful insights into the issues to be considered when structuring and implementing such reviews, they are unlikely to find it necessary or feasible to follow all of the recommended steps.

### 3.2 Evaluation Criteria

Perhaps the most important components of the above approach are the criteria used to evaluate the quality and applicability of the related research. The starting point for developing these criteria was the HHS *Guidelines*. We supplemented these guidelines with information from several articles and texts that discuss best practices, as cited throughout this report, as well as with what we have learned from our own experience in conducting related analyses.<sup>19</sup>

Exhibit 1 summarizes the criteria, which we describe in more detail below. This discussion provides an overview of related issues; analysts should review the references cited and other resources, as well as consult with costing experts familiar with the conditions of concern, to develop a more detailed understanding of these issues.

#### Exhibit 1: Evaluation Criteria

##### General Criteria

1. Addresses the U.S. population.
2. Includes needed cost elements.
3. Publicly-available and well-documented.

##### Quality Criteria

4. Independently reviewed.
5. Estimates social opportunity costs.
6. Uses appropriate analytic methods.

##### Applicability Criteria

7. Relies on recent data.
8. Addresses individuals with similar characteristics.
9. Addresses similar health conditions.

When developing medical cost estimates, analysts will often be able to choose between relying on available research studies or retrieving estimates from available databases, such as the Medical Expenditure Panel Survey (MEPS) or Healthcare Cost and Utilization Project (HCUP). We refer to “studies” in the discussion that follows for convenience. However, the same criteria should be applied to the available databases.

---

<sup>19</sup> The books and articles we identified that discuss best practices generally address the estimation of medical costs in cost-of-illness studies (e.g., the series of articles in Yabroff et al. 2009a) or in cost-effectiveness analyses (e.g., Neumann et al. 2016) rather than in benefit-cost analysis.

## General Criteria

The first three criteria relate to the context within which the estimates will be applied.

**Criterion 1: Addresses the U.S. population.** Given the significant variation in health care systems and financing across countries, only studies that estimate costs in the U.S. should be considered.

**Criterion 2: Includes needed cost elements.** The medical cost components needed will depend on the regulatory context, as discussed under Step 1 in Section 3.1. If the estimates will supplement the benefit values, then studies must provide estimates of costs paid by insurance or other third parties.<sup>20</sup> If the estimates will be used to assess regulatory costs, then the studies must address those costs affected by compliance with the particular regulation. In some cases, the regulation may affect the full costs of per patient care, including associated overhead; in other cases the regulation may only affect some cost components.<sup>21</sup> The studies should provide a reasonably complete accounting of the relevant costs, while allowing the analyst to exclude unaffected costs.

**Criterion 3: Publicly-available and well-documented.** Decision-makers and other stakeholders must be able to review the data sources used to support the regulatory analysis, and hence must be able access the study. The data and methods used, and the results and associated uncertainties, should be clearly documented.

## Quality Criteria<sup>22</sup>

The next three criteria relate to the quality of the study, including the consistency of the estimates with the conceptual framework for benefit-cost analysis.

**Criterion 4: Independently reviewed.** While publication in a peer-reviewed journal or in a report issued or reviewed by an expert panel provides substantial evidence of quality, such reviews do not necessarily address the issues of interest to regulatory analysts. For example, peer-reviewed journals may focus on whether the work is innovative, rather than on issues such as whether the sample is representative. Thus while articles published in peer-reviewed journals and expert panel reports generally meet this criterion, analysts should supplement these reviews with further evaluation of the quality of the work as discussed under the following criteria. Work that has not yet undergone such independent expert review may be considered, but requires particularly careful scrutiny by analysts.

**Criterion 5: Approximates social opportunity costs.** For benefit-cost analysis of Federal regulations, medical cost estimates would ideally reflect the opportunity costs associated with the changes in the

---

<sup>20</sup> As noted earlier, some proportion of insured medical costs will be covered by the patient, due to deductible and copayment requirements. Given variation in these payments across insurance plans and patients, it may not be possible to separate out such costs. Related uncertainties should be clearly communicated in presenting the analytic results.

<sup>21</sup> If the regulation requires changes in current medical treatment practices that would not occur in the absence of regulation, then the associated investments in research and development may need to be considered. See also the discussion of the analytic time frame under Criterion 7.

<sup>22</sup> We thank Dr. David Rein of NORC for his substantial contributions to the development of Criteria 5 and 6.



use of real resources nationally; i.e., on a society-wide basis. However, such costs are difficult to define and measure in a health care context, due to significant market distortions. Analysts will need to carefully review the available data sources, measures, and methods to determine which best approximate these values, and should discuss the limitations of the available data in presenting the analytic results. Often, expenditures represented as actual reimbursements, expected reimbursements from payment schedules, or cost-adjusted charges are likely to be reasonable measures, as long as they represent the costs imposed on a typical payer (see, for example, Larg and Moss 2011).

More generally, several alternative concepts are used to measure health care costs in the literature, including: (1) micro-costing; (2) total payments; (3) allowable charges; (4) cost-adjusted charges; and (5) unadjusted charges (see, for example, Onukwugha et al. 2016). We summarize these approaches in Exhibit 2 then discuss their relationship to opportunity costs in more detail. While each of these approaches can be feasibly implemented, even the best of these methods lead to somewhat uncertain estimates of opportunity costs, given the challenges described in Section 2.1.

**Exhibit 2: Approaches for Measuring Health Care Costs**

<b>Approach</b>	<b>Description</b>	<b>Strengths and Limitations</b>	<b>Quality</b>
1. Micro-costing	Sums the value of labor, facilities, supplies, insurance, and capital inputs.	<ul style="list-style-type: none"> <li>• Best available estimate of actual production costs.</li> <li>• Costly and time consuming to collect.</li> </ul>	Good
2. Total Payments	Sums insurance reimbursement, copayment, deductible, and coordination of benefits amounts regardless of in-network or out-of-network status.	<ul style="list-style-type: none"> <li>• Best available proxy for actual production costs.</li> <li>• May exclude obligated payments written off by the provider.</li> <li>• May include costs from only a single institution or payer.</li> </ul>	Good
3. Allowable Charges	Maximum amount an insurer is willing to pay for a service, including costs borne by the patient.	<ul style="list-style-type: none"> <li>• Reasonable proxy; includes actual production costs for in-network providers but excludes out-of-network costs.</li> <li>• May include costs from only a single institution or payer.</li> </ul>	Moderate
4. Adjusted Charges	Provider service charges adjusted for provider or service area cost-to-charge ratios.	<ul style="list-style-type: none"> <li>• Reasonable proxy for production costs; often only estimate available.</li> <li>• Cost-to-charge ratios may vary considerably within a provider or service area.</li> </ul>	Fair
5. Unadjusted Charges	Provider-posted asking price.	<ul style="list-style-type: none"> <li>• Does not reflect opportunity costs; can vary widely based on idiosyncratic factors.</li> </ul>	Poor; should not be used.

In addition to reviewing the approach used, analysts should consider how well the approach is implemented. The advantages of applying a less well-conducted study that uses highly-ranked approach will need to be weighed against the advantages of applying a more carefully-conducted study that uses a

lower-ranked approach. These advantages and limitations should be documented when discussing the rationale for selecting particular studies and the uncertainties in the estimates.

*Micro-costing* refers to studies that collect data from patients or providers on services required for providing and managing care, regardless of whether the services are coded and claimed for reimbursement (see, for example, Farre et al. 2016). Researchers using this method must be able to observe full episodes of condition care so as to estimate the total quantity of different types of services (outpatient visits, lab tests, hospitalization days, etc.) (Ruger and Reiff 2016). The goal of micro-costing is to identify all such services utilized in an average episode of care and then estimate the production cost of each, primarily as a function of labor, supply, capital, and other cost inputs (Jacobs and Barnett 2017). In some instances, a micro-costing study will only collect data on the quantity of services consumed and in a subsequent step apply unit cost information taken from other sources.

Such studies are often the best source for approximating opportunity costs because they include all the incremental costs of providing a service, not only those that are reimbursed.<sup>23</sup> However, researchers must take care when collecting information to avoid problems related to recall bias, and these studies often rely on very small samples. In addition, micro-costing studies are unable to directly measure overhead costs. Like other types of research, cost-collection studies should be evaluated based on the quality of their data collection instruments and methods.

*Total (aggregate) payments* refers to the sum of payments reported in administrative databases, such as those used to calculate reimbursements (Riley 2009). Ideally, these amounts would include payments for both in-network and out-of-network providers across a range of providers and insurance plans – assuming the regulation’s effects are national rather than limited to particular providers or insurers. Common variables include insurance payments, patient copayments, deductibles, and coordination of benefits (COB, i.e., payments from other insurers) for each submitted claim. In some sources, administrative claims data will include a “total payments” variable summing the values for each possible payment category. When all these fields are available, the sum is used to estimate the actual payments a provider received for each service rendered. In some cases, estimates based on these total payments may be preferable to estimates from micro-costing studies; for example, if the goal is to estimate the cost of a condition to an insurance system such as Medicaid. However, these studies do not capture services that are excluded from insurance payments and some administrative databases may not include all needed variables. In general, however, such studies are a good source of cost information.

*Allowable charges* differ from total payments in that they reflect the amount an insurance company has determined to be the appropriate payment for a service. They do not include amounts directly charged to patients for out-of-network providers or services, so are not likely to fully capture the national per

---

<sup>23</sup> As noted earlier, whether costs paid by patients should be included in estimates developed to support regulatory benefit-cost analyses will depend on (1) whether such costs are affected by the regulation, and (2) whether the medical cost estimates are included in the estimation of regulatory “costs” or “benefits.” In the latter case, patients’ out-of-pocket costs may be included in the WTP measures used to value mortality and morbidity risk reductions, and the potential for double-counting should be explicitly considered and discussed.

patient costs for the condition. However, research has found them to serve as a good proxy measure for the standard costs incurred by most patients (Schousboe et al. 2016).

While allowable charges include the total amount the provider is entitled to recoup, the allocation between the insurer and the insured will depend on deductibles, copayments, and annual out-of-pocket maximums, which can vary by patient and by when the charge is incurred as well as by plan. Allowable charges are equal to the full payment for the service when the insurer has a contractual relationship with the provider; for example, through a preferred provider organization. However, they exclude additional charges a patient may be obligated to pay for out of network services, or by patients who are uninsured (Bai and Anderson 2015).

Allowable charges may not account for all of the opportunity costs of a service for other reasons as well. Providers contract with insurers for multiple services and often lack the bargaining power needed to ensure that their full costs are covered for all services from all insurers. Insurers do not have the full information needed for appropriate pricing and may over- or under-pay for some services. Allowable charges vary across insurers and also may not fully capture the obligated payments for services delivered by out-of-network providers. Thus even if a provider recoups its costs in total (across all conditions and services), the allocation of costs to a particular condition may be imprecise for these and other reasons.

*Adjusted charges* involves modifying reported charges when payments or allowable charge data are not available. In this case, provider charges are adjusted by an average cost-to-charge ratio; i.e., an estimate of the provider's (usually a hospital's) total costs, as reported to the Centers for Medicare and Medicaid Services (CMS) through their Medicare Cost Report, divided by their total charges (see, for example, Skolasky and Riley 2015). Although adjusted charges are superior to unadjusted charges for measuring opportunity costs, they are inferior to total payments or allowable charges because (except in specialized studies) only a single cost-to-charge ratio is available while actual cost-to-charge ratios can vary substantially across service categories within a hospital or hospital system. In addition, the quality of the estimates can vary greatly based on the quality as well as the applicability of the cost-to-charge ratio used.

*Unadjusted charges* from providers should not be used to measure opportunity costs. They are generally misleadingly inflated and vary widely between providers and across services; analysts should avoid using such studies in regulatory analysis. In some cases, analysts may be able to adjust these charges (e.g., using the Medicare fee schedule) to better reflect actual payments or to transfer an estimate that uses one of the above methods for a similar condition. If unadjusted charges are the only practical option, analysts should exercise caution. For example, these charges could be included in a sensitivity analysis rather than in the primary estimate of regulatory benefits or costs, and discussed in the section that describes the uncertainties in the estimates of net benefits. In most circumstances an analyst is likely to encounter, alternative estimates based on more accurate cost data should exist which will negate the need to consider estimates based on unadjusted charge data.

The estimation of opportunity costs for drugs and devices is particularly controversial (see Garrison et al. 2010, Hay et al. 2010), given that the incremental costs of producing an additional unit are often small compared with the costs of developing and obtaining regulatory approval for the product. For example, some argue that these values should reflect only the short run marginal cost of producing and distributing a drug, whereas others argue that the long run costs of research and development and of seeking regulatory approval should also be included. We do not attempt to resolve this controversy here; however, analysts should be aware of these concerns and explore the sensitivity of the results to different approaches, especially when drugs or devices – or other treatments that require substantial innovation -- are a significant proportion of the overall medical costs. Note that the recently released report of the Second Panel on Cost-Effectiveness in Health and Medicine (Neumann et al. 2016) recommends the use of the Federal Supply Schedule for drug pricing.

**Criterion 6: Uses appropriate analytic methods.** As introduced earlier, within the context of a regulatory analysis, the goal is to estimate the net change in medical costs that is attributable to the regulation. However, different methodological approaches can lead to widely varying cost estimates (see, for example, Barlow 2009, Coughlan et al. 2014, Yabroff et al. 2009b,c). In addition, costing articles may not include sufficient information on how unit costs were calculated. Other difficulties include capturing the direct and indirect effects of conditions on patient costs, especially because some services such as hospital admissions may address several comorbidities, and services may not always be identifiable by diagnosis code.

Three methods dominate the medical cost literature as summarized in Exhibit 4 and discussed below: (1) econometric or regression-based methods; (2) accounting methods; and (3) cost-modeling based on (a) practice patterns or (b) expert opinion.<sup>24</sup> Note that in discussing the relative quality of these methods, we assume that the studies are equally well-conducted. Otherwise, a study that uses a lower ranked method may be preferable to study that uses a higher ranked method if the latter is poorly conducted. Analysts should review the references cited in this report and elsewhere, and consult with experts in the use of these methodologies, to evaluate the extent to which individual studies adhere to established best practices.

---

<sup>24</sup> For more discussion of the advantages and limitations of these and other approaches, see, for example, Clabaugh and Ward (2008), Larg and Moss (2011), and the collection of articles in Yabroff et al. (2009), as well as the references cited throughout the text. General health economics texts, such as Drummond et al. (2015) are also useful.

**Exhibit 4: Cost Estimation Methods**

<b>Method</b>	<b>Description</b>	<b>Strengths and Limitations</b>	<b>Quality</b>
1. Econometric or regression-based methods	Statistical estimation of the incremental cost of having a condition compared to not having a condition, controlling for confounding characteristics.	<ul style="list-style-type: none"> <li>• Best source of cost estimation when confounding can be controlled.</li> <li>• May over- or under-estimate costs when influential patient characteristics are unobserved and hence not captured in the data.</li> </ul>	Good
2. Accounting methods	Average per patient costs associated with claims specifically coded for the condition.	<ul style="list-style-type: none"> <li>• May provide a lower bound estimate of costs.</li> <li>• May exclude incremental increases in services that are not directly coded for a condition.</li> </ul>	Moderate
3(a). Cost modeling based on guidelines	Published care guidelines are translated into specific services that are coded in claims data and assigned costs based on published fee schedules.	<ul style="list-style-type: none"> <li>• Provides an estimate of costs of ideal care based on consensus statements of quality treatment.</li> <li>• Actual care may differ substantially from ideal care.</li> </ul>	Fair
3(b). Cost modeling based on expert opinion	Author or expert opinions regarding care are translated into costs based on published fee schedules.	<ul style="list-style-type: none"> <li>• May be the only estimate available for rare conditions that lack treatment guidelines or data.</li> <li>• Lack evidence base to link to actual care patterns or consensus statements to costs.</li> </ul>	Poor

*Econometric or regression-based methods* refer to the use of statistical methods to estimate the incremental costs associated with a condition (usually per year or per month), controlling for other differences between individuals who do and do not experience the condition. In econometric approaches, costs are modeled as a function of a dichotomous indicator of the condition of interest and a series of independent variables. These independent variables are intended to control for patient-level differences related to health care costs that may be correlated with, but not caused by, the condition of interest. When such confounding characteristics are properly controlled, econometric methods can capture the full incremental costs of a condition across all health expenditures over the time period as compared to not having the condition (assuming the measure of costs used as the dependent variable is appropriate). A variation on econometric methods involves quasi-experimental matching of patients based on baseline characteristics observable prior to a diagnosis, with matching determined via an algorithm (pairwise, coarsened exact matching) or via a separate logistic regression used to create a propensity (similarity) score (see, for example, Li et al. 2016). Matched samples can be compared with or without using regression to control for other independent variables to determine the average incremental cost of a condition (see, for example, McAdam-Marx et al. 2011).

As discussed under Step 1 in the previous section, the types of medical costs affected by a regulation vary and do not necessarily include all costs attributable to an incident case. Thus analysts will need to take care to ensure that the methods used address the appropriate cost elements. Statistical models can

compare costs without and with the regulation using either econometric equations that control for differences in patient characteristics, quasi-experimental matching techniques, or both, much in the same way a clinical trial would use an experimental design to estimate cost differences between those who did and did not receive a treatment (see, for example, Basu and Manning 2009).

*Accounting methods* refer to the process of identifying all claims associated with a condition of interest (using those claims coded with an appropriate diagnosis code), summing the payments or allowable charges associated with each claim over a specific interval (usually a year but sometimes an algorithmically-defined episode of illness). The mean cost per patient is then estimated across all the patients with a condition. While this method generally captures the costs of services directly related to a condition, it typically misses those costs associated with either direct services lacking a diagnosis code or services that are indirectly affected (positively or negatively) by the presence of the condition. Accounting cost estimates may be preferable to econometric estimates if controlling for patient-level confounders is confusing or impossible given data limitations.

*Cost-modelling methods* refer to developing a cost model by converting a model of patient treatment into a list of specific medical services, procedures, and tests; applying estimates of the costs of each service, procedure, or test to these cost components; and then aggregating the modeled cost components. In most cases, component costs are estimated using the Medicare reimbursement rate, which is equivalent to the allowable charge applicable within the Medicare system. As noted earlier, the goal of regulatory analyses is to provide estimates of benefits and costs that are as realistic as possible; the extent to which such models reflect actual “real world” medical costs depends on whether they are based on ideal or observed practices.

These models may be derived from published treatment guidelines or preferred practice patterns, which may differ from actual practices. Once a treatment model has been developed, the services named in the model are converted into current procedural terminology (CPT) codes or healthcare common procedure coding system (HCPCS) codes, and costs associated with each code are taken from Medicare reimbursement schedules or other sources. Costs across these services are then summed by illness category usually to estimate a cost per treatment stage. In more advanced models, these costs can be combined with probabilities of disease stage occurrence to estimate either a weighted average cost of treatment across all stages in any given year, or the estimated lifetime cost of an incident case accounting for the probabilities and costs of future disease progression (a method that can be applied regardless of the source of cost inputs).

Cost-modelling provides a very specific cost result: the estimated cost of providing care that complies perfectly with the treatment model, which may reflect expected care, normal care, or ideal care. As such, it can be thought of as an estimate of the direct costs of the type of care without accounting for additional indirect effects of the illness on other services. Thus it often does not capture some incremental costs or cost offsets, and care that perfectly adheres to a treatment model rarely occurs in clinical practice.

Alternatively, rather than relying on established guidelines, cost modeling may be implemented by substituting the opinions of researchers or other experts regarding the appropriate care for a more formal, process-based model of patient treatment. As before, the elements of care are translated into costs based on estimates of each assumed component of the care process. While such models may be the only estimate available for some conditions, the lack of evidence on actual or recommended care patterns is problematic.

The appropriateness of these approaches needs to be considered in tandem with the appropriateness of the cost measure used, as discussed under Criterion 5. Whether a high quality approach that uses a lower quality measure of opportunity costs should be applied in a particular regulatory analysis will depend on the other options available, as well as the extent to which each study meets the other evaluation criteria.

### **Applicability Criteria**

The three final criteria relate to the applicability of the estimates; each criterion encompasses several considerations.

***Criterion 7: Relies on recent data.*** Given the continuing evolution of the health care system as well as changes in the overall economy, data that reflect the resource costs associated with current and potential future medical practices are desirable.<sup>25</sup> However, analysts will generally need to rely on data collected in previous years. While a more-recently conducted study is preferable to an older study, all else equal, it is not possible to define a fixed cut-off, especially since the degree of change over time will depend on the condition of interest as well as the time period.

Generally, studies that rely on data collected within the past 10 years are preferable; studies that rely on data that are more than 20 years old should be avoided. Note that this criterion refers to the date when the costs were actually incurred, not the date when the study was published, which may be several years later.

When initiating the literature review, analysts should consult with relevant experts to determine whether medical care for the condition(s) of concern has experienced substantial innovation in recent years or has changed dramatically for other reasons. In such cases, analysts may need to limit the review to articles that rely on more recent data, retrieve data themselves from available data bases (such as MEPS or HCUP), or work with medical costing experts to adjust the available estimates to reflect current practices or to design appropriate analyses of uncertainty. To the extent possible, analysts should also consider the extent to which major changes in medical costs are likely over the time period covered by the analysis which may affect the incremental impacts of the regulation. Related uncertainties should also be addressed in discussing the analytic results.

---

<sup>25</sup> This criterion refers to the resource costs associated with changing medical practices, not to the effects of inflation. Adjustments for inflation are discussed in the HHS Guidelines and OMB (2017).

**Criterion 8: Addresses similar population.** Applying this criterion involves considering the relationship of the population studied to the population affected by the regulation. As noted under Criterion 1, only studies conducted in the U.S. should be included in the review. Criterion 8 refers to the specific subgroups affected, who may differ significantly from the average member of the overall U.S. population.

- Personal characteristics: These include factors such as age, underlying health status, geographic location, and income, which may affect the types of medical care received as well as its costs and effectiveness.
- Sample characteristics: A probabilistic sample is preferred to a convenience sample, because it is more likely to be representative of the population. The size of the sample should be sufficient to adequately represent the population and to provide estimates of reasonable precision. The adequacy of a particular sample size will depend on the analytic methods used and on the variance of the parameter that is to be estimated.
- Comparison group: As noted earlier, regulatory analysis involves comparing predicted future conditions without the regulation to conditions with the regulation. Thus the appropriate comparison for medical costs is often between a person with the condition and an otherwise identical person without the condition. However, if the regulation affects the treatment of a condition rather than its incidence, then the appropriate comparison is between otherwise identical people who receive the two forms of treatment.

**Criterion 9: Addresses similar health condition.** Applying this criterion involves considering factors such as the following:

- Severity of the condition and the effects of associated treatments.
- Duration, in particular whether the condition persists for less than a year or for a longer time span. Many medical cost studies consider impacts for only a single year, which may be appropriate for short-lived conditions. For chronic conditions, which may persist for many years or the remainder of one's lifespan, longitudinal data are desirable, but primary data sources may cover past periods when treatment options and recovery rates may have been different from today. In such cases, simulation models are usually needed to estimate long-term costs.

#### **4.0 SUMMARY AND CONCLUSIONS**

Developing medical cost estimates for use in regulatory benefit-cost analyses poses many challenges, given the issues raised by the underlying conceptual framework and the databases and research currently available. The approach in this report suggests best practices to encourage consistency across HHS analyses as well as efficient use of analytic resources. It consists of four steps: (1) clearly define the condition to be addressed; (2) conduct a criteria-driven literature review; (3) identify research gaps and options for addressing them; and (4) implement selected approach and address uncertainty.



Perhaps the most important component of this process is developing criteria to be used in evaluating the literature. We suggest nine criteria that address the general context for applying the estimates, the quality of the data, and the applicability of the data to the population and health conditions to be addressed. These criteria include: (1) addresses the U.S. population; (2) includes needed cost elements; (3) publicly-available and well-documented; (4) independently reviewed; (5) estimates social opportunity costs; (6) uses appropriate analytic methods; (7) relies on recent data; (8) addresses individuals with similar characteristics; (9) addresses similar health conditions. Typically, no individual study will meet all of the criteria. Deciding whether to apply a study in a regulatory analysis thus requires judgment on the part of the analyst; analysts should describe the extent to which selected studies meet these criteria to aid reviewers in understanding the basis for their choices as well as the advantages and limitations of the approach.

## REFERENCES

- Bai, G. and G. F. Anderson. 2015. "Extreme Markup: The Fifty US Hospitals With The Highest Charge-To-Cost Ratios." *Health Affairs*. 34(6): 922-928
- Barlow, W.E. 2009. Overview of Methods to Estimate the Medical Costs of Cancer." (2009): *Medical Care*. 47(7): Supplement 1: S33-36.
- Basu, A. and W. G. Manning. 2009. Issues for the Next Generation of Health Care Cost Analyses. *Medical Care*. 47(7): Supplement 1: S109-114.
- Baxter, J.R., L.A. Robinson, and J.K. Hammitt. 2017. *Valuing Time in U.S. Department of Health and Human Services Regulatory Impact Analyses: Conceptual Framework and Best Practices*. Prepared by Industrial Economics, Incorporated for the U.S. Department of Health and Human Services.
- Bloom, B.S. et al. 2001. "Usefulness of U.S. Cost of Illness Studies in Healthcare Decision Making." *Pharmacoeconomics*. 19(2): 207-213.
- Clabaugh, G. and M.M. Ward. 2008. "Cost-of-Illness Studies in the United States: A Systematic Review of Methodologies Used for Direct Cost." *Value in Health*. 11(1): 13-21.
- Clinton, W.J. 1993. "Executive Order 12866: Regulatory Planning and Review." *Federal Register*. 58(190): 51735-51744. <https://www.archives.gov/files/federal-register/executive-orders/pdf/12866.pdf>
- Coughlan, D. et al. 2014. "Evaluating Direct Medical Expenditures Estimation Methods of Adults Using the Medical Expenditure Panel Survey: An Example Focusing on Head and Neck Cancer." *Value in Health*. 17(1): 90-97.
- Drummond, M.F., et al. 2015. *Methods for the Economic Evaluation of Health Care Programmes, 4rd Edition*. Oxford, U.K.: Oxford University Press.

- Farre, M. et al. 2016. Direct and Indirect Costs and Resource Use in Dementia Care: A Cross-Sectional Study in Patients Living at Home. *International Journal of Nursing Studies*. 55: 39-49.
- Garrison, L.P. et al. 2010. "Good Research Practices for Measuring Drug Costs in Cost-Effectiveness Analyses: A Societal Perspective: The ISPOR Drug Cost Task Force Report—Part II." *Value in Health*. 13(1): 8-13.
- Hay, J.W. et al. 2010. "Good Research Practices for Measuring Drug Costs in Cost-Effectiveness Analyses: A Societal Perspective: The ISPOR Drug Cost Task Force Report—Part I." *Value in Health*. 13(1): 3-7.
- Hirth, R. A. et al. 2000. "Willingness to Pay for a Quality-Adjusted Life Year: In Search of a Standard." *Medical Decision Making*. 20: 332-342.
- Honeycutt, A.A. et al. 2009. "Comparing Cost-of-Illness Estimates from Alternative Approaches: An Application to Diabetes." *Health Services Research*. 44(1): 303-320.
- Institute of Medicine. 2011. *Finding What Works in Health Care: Standards for Systematic Reviews*. Washington, DC: National Academies Press.
- Jacobs, J. C. and P. G. Barnett. 2017. "Emergent Challenges in Determining Costs for Economic Evaluations." *Pharmacoeconomics*. 35: 129-139.
- Larg, A. and J.R. Moss. 2011. "Cost-of-Illness Studies: A Guide to Critical Evaluation." *Pharmacoeconomics*. 29(8): 653-671.
- Li, J. et al. 2016. "Propensity Score and Doubly Robust Methods for Estimating the Effect of Treatment on Censored Cost." *Statistics in Medicine*. 35(12): 1985-1999
- Lund, J.L. et al. 2009. "Inventory of Data Sources for Estimating Health Care Costs in the United States." *Medical Care*. 47(7, Supp. 1): S127-S142.
- McAdam-Marx, C. et al. 2011. "All-Cause and Incremental per Patient per Year Cost Associated with Chronic Hepatitis C Virus and Associated Liver Complications in the United States: A Managed Care Perspective." *Journal of Managed Care Pharmacy*. 17(7): 531-546
- Moher, D. et al. 2009. Preferred Reporting Items for Systematic Reviews and Meta-Analyses: The PRISMA Statement. *PLoS Medicine*. 6(7): e1000097.
- Neumann, P.J. et al. (eds.), 2016. *Cost-Effectiveness in Health and Medicine* (Second Edition). New York: Oxford University Press.
- Obama, B. 2011. "Executive Order 13563: Improving Regulation and Regulatory Review." *Federal Register*. 76(14): 3821-3823. <https://www.gpo.gov/fdsys/pkg/FR-2011-01-21/pdf/2011-1385.pdf>

- Onukwugha, E. et al. 2016. Cost-of-Illness Studies: An Updated Review of Current Methods. *Pharmacoeconomics*. 34(1): 43-58.
- Riley, G. F. 2009. "Administrative and Claims Records as Sources of Health Care Cost Data." *Medical Care*. 47(7, Supp. 1): S51-S55.
- Robinson, L.A. and J.K. Hammitt. 2015. Introduction to the Special Series: Research Synthesis: A Cross-Disciplinary Approach." *Risk Analysis*. 35(6): 963-970.
- Robinson, L.A., J.K. Hammitt, A.Y. Chang, and S. Resch. 2017. "Understanding and Improving the One and Three Times GDP per Capita Cost-Effectiveness Thresholds." *Health Policy and Planning*. 32: 141-145.
- Ruger, J. P. and M. Reiff. 2016. A Checklist for the Conduct, Reporting, and Appraisal of Microcosting Studies in Health Care: Protocol Development. *JMIR Research Protocols*. 5(4): e195.
- Russell, L.B. 2009. Completing Costs: Patients' Time. *Medical Care*. 47(7) Supplement\_1: S89-S93.
- Sanders, G.D. et al. 2016. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-Effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 316(10): 1093-1103.
- Schousboe, J. T. et al. 2016. "Estimating True Resource Costs of Outpatient Care for Medicare Beneficiaries: Standardized Costs versus Medicare Payments and Charges." *Health Service Research*. 51(1): 205-219.
- Skolasky, R. L. and L. H. Riley. 2015. "Medicare Charges and Payments for Cervical Spine Surgery: Association with Hospital Characteristics." *Spine*. 40(16): E936-942.
- Trump, D. 2017. "Executive Order 13771: Reducing Regulation and Controlling Regulatory Costs." *Federal Register*. 82 (22): 9337-9341. <https://www.federalregister.gov/documents/2017/02/03/2017-02451/reducing-regulation-and-controlling-regulatory-costs>
- U.S. Department of Health and Human Services. 2016. Guidelines for Regulatory Impact Analysis. Prepared for the Office of the Assistant Secretary for Planning and Evaluation by L.A. Robinson, J.K. Hammitt, and J.R. Baxter under subcontract to Industrial Economics, Incorporated and Mathematica Policy Research.
- U.S. Office of Management and Budget. 2003. *Circular A-4: Regulatory Analysis*. <https://www.federalregister.gov/documents/2003/10/09/03-25606/circular-a-4-regulatory-analysis>
- U.S. Office of Management and Budget. 2016. *Draft Report to Congress on the Benefits and Costs of Federal Regulations and Agency Compliance with the Unfunded Mandates Reform Act*. [https://obamawhitehouse.archives.gov/sites/default/files/omb/assets/legislative\\_reports/draft\\_2016\\_cost\\_benefit\\_report\\_12\\_14\\_2016\\_2.pdf](https://obamawhitehouse.archives.gov/sites/default/files/omb/assets/legislative_reports/draft_2016_cost_benefit_report_12_14_2016_2.pdf)

U.S. Office of Management and Budget. 2017. "Implementing Executive Order 13771, Titled 'Reducing Regulation and Controlling Regulatory Costs.'" Memorandum for Regulatory Policy Officers at Executive Departments and Agencies and Managing and Executive Directors of Certain Agencies and Commissions, from Dominic J. Mancini, Acting Administrator, Office of Information and Regulatory Affairs. M-17-21 <https://www.whitehouse.gov/the-press-office/2017/04/05/memorandum-implementing-executive-order-13771-titled-reducing-regulation>

Yabroff, K.R. et al. (eds.). 2009a. Health Care Costing: Data, Methods, Future Directions. *Medical Care*. 47(7): Supplement 1.

Yabroff, K.R, et al. 2009b. Comparison of Approaches for Estimating Incidence Costs of Care for Colorectal Cancer Patients. *Medical Care*. 47(7): Supplement 1: S56-S63.

Yabroff, K. R., et al. 2009c. Comparison of Approaches for Estimating Prevalence Costs of Care for Cancer Patients: What is the Impact of Data Source?" *Medical Care*. 47(7): Supplement 1: S64-69.