

# Key Questions for Legislators about the Institute of Clinical and Economic Review (ICER)

By Dr. William S. Smith



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## Table of Contents

Introduction	4
The Emergence of QALY	4
NICE Controversies and the Use of QALY	4
ICER and the United States	4
ICER and American Political Culture	5
Questions about the Potential Limitations of the ICER Model	6
Ethical Considerations	6
Methodological Issues and Theoretical Assumptions	7
Contextual or Condition-Specific Considerations	9
Conclusion	9



## Introduction

The costs of prescription drugs are a prominent issue for state and federal legislators across the nation. Research and development trends in the pharmaceutical industry are yielding more and more therapies for rare and orphan diseases, therapies that typically have much higher prices than traditional small molecule drugs. While the economics of drugs for rare diseases are well established, i.e. drugs that target a smaller pool of patients with very serious diseases tend to be much more expensive, some policy makers have expressed “sticker shock” at the prices of certain therapies and are exploring options that may lower prices.

## The Emergence of QALY

One strategy that policy makers are considering is the adoption of so-called “cost-effectiveness” reviews that purportedly represent an objective method of evaluating whether particular therapies are over priced or “cost effective.” The National Institute for Health and Care Excellence (NICE) in the United Kingdom (UK) developed the most prominent methodology for cost effectiveness reviews beginning in the 1970s. NICE’s methodology utilizes the “Quality Adjusted Life Year” (QALY) standard which assigns a monetary value to the quality of life and survival length for patients and then assesses the cost effectiveness of a drug based upon the drug’s potential ability to both improve a patient’s quality of life and to extend that life.

While a number of aspects of the NICE model have generated controversy, many criticisms flow from the use of the QALY standard. One QALY equals one full year of life in perfect health. The value of one QALY is assigned a monetary value; in the case of the

Institute of Clinical and Economic Review (ICER), one year of perfect health is valued at about \$100,000–\$150,000. The QALY therefore combines into a single cost index the combined value of the length and quality of life. When patients need a certain therapy, the QALY model assigns a value to the therapy

based upon how long it would prolong life and how much it would raise the quality of life. Questions about the validity of using the QALY standard are discussed below.

The use of QALY has generated worldwide controversy, with nations such as the UK, Canada and Australia adopting QALY measurements, while the United States and Germany have rejected their use. For example, one major health outcomes [study](#) of the 27 European health systems conducted by

the European Consortium in Healthcare Outcomes and cost benefit research concluded that, “QALY assessment for health decision making should be abandoned.”

## NICE Controversies and the Use of QALY

NICE’s methodology has generated significant criticism in the United Kingdom from both patient advocates and physicians, as well as from the biopharmaceutical industry for two reasons. First, therapies are typically denied to patients in the National Health Service until a NICE review has certified that these new drugs are “cost effective.” Second, even once NICE reviews are complete, many therapies are deemed as not cost effective and denied to patients. There is some [evidence](#) that NICE’s reviews have made the latest pharmacopeia less available to British patients than to patients in other nations.

Some of the most intensive criticism of NICE’s methodology has come from oncology, where critics have noted that delays in NICE reviews were, quite literally, causing the deaths of patients who were awaiting reviews of new cancer treatments that were already widely available in other nations. To circumvent the NICE review process, in April of 2010, then-Prime Minister David Cameron announced the creation of a “Cancer Drugs Fund” that would fund cancer treatments regardless of the conclusions of a NICE cost effectiveness review. In announcing the Cancer Drugs Fund, Cameron [said](#):

Other European countries are doing better than us at giving people longer, happier lives with cancer. We want to get more drugs to people more quickly and in the UK today there are some people – thousands of people – who want a certain cancer drug, whose doctors tell them they should have a certain cancer drug, who don’t get it.

The origin of the Cancer Drugs Fund raises some of the most serious issues surrounding the use of the QALY standard in assessing the value of therapies for dreaded diseases. It seems clear that the people of the United Kingdom, and their elected officials, were not comfortable with the monetary value assigned to human life by the QALY standard, as it resulted in the denial of life-saving drugs available in many other countries.

## ICER and the United States

In the United States, ICER has adopted a cost effectiveness methodology similar to NICE that also utilizes the QALY standard. Despite the intense criticism in the UK that this methodology has led to drug rationing, some US policy makers are considering using ICER’s conclusions for structuring government drug formularies. For example, the New York State Medicaid program [utilized](#) an ICER review to evaluate whether to pay for Orkambi, a breakthrough treatment for cystic fibrosis.

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The pharmacy benefit management company CVS Caremark also recently [announced](#) that they would begin utilizing ICER data for their formulary management and would establish a hard QALY cap of \$100,000; i.e. if a drug could not prove a value above \$100,000, it would not be covered. CVS's use of ICER data is particularly controversial because if the company were to succeed at lowering a drug's price based upon an ICER review, the reduction in price would likely take the form of a higher rebate payment from the drug manufacturer that would flow to CVS itself, not necessarily a reduction in the patient's out-of-pocket cost.

Finally, even the federal government may be considering the inclusion of some type of cost effectiveness reviews for Medicare and Veterans Administration formularies. The VA has already established a [partnership](#) with ICER. While the Affordable Care Act bans the use of QALY in developing a Medicare formulary, the Centers for Medicare and Medicaid (CMS) recently announced that Medicare Part D plans could begin adopting "indication-based" criteria for their drug formularies in 2020.

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health policy experts have [concluded](#) that CMS's adoption of indication-based formularies portends the adoption of cost effectiveness studies, such as the use of ICER reviews, when assembling drug formularies for older Americans and the disabled.

## ICER and American Political Culture

U.S. policy makers have a genuine budgetary challenge in addressing high-cost treatments for patients with serious diseases. However, ICER cost effectiveness reviews seem particularly controversial for older patients, those with disabilities, cancer patients, and patients with rare diseases. Given these controversies, policy makers may want to tread thoughtfully and carefully. It seems unlikely that the American public will accept the kinds of rationing of therapies and medical services that British political culture, with 70 years of socialized medicine, has largely come to accept. The political firestorm in the US would likely be far greater than it was in the UK if U.S. senior citizens were denied new oncology treatments while the federal government conducted cost effectiveness reviews. The political convulsion would probably be even more pointed if that review were to conclude that U.S. seniors should be

denied a new oncology treatment that was available to citizens in other countries.

A similar political backlash may occur in state Medicaid programs that serve as a safety net for many of the very patients suffering from rare and debilitating diseases. Many patients in Medicaid and in the prison system do not currently have access to many of the latest treatments. Would advocates for the poor permit even deeper restrictions on access to medications for these populations if ICER reviews made such recommendations? Again, the American public may be less accepting than the British public if a disabled child in the Medicaid program were denied a lifesaving or life altering therapy because of an ICER review.

For the New England area, and the Boston-Cambridge area in particular, the adoption of cost effectiveness methodologies also could have economic development consequences as the region contains a robust cluster of biopharmaceutical companies that are the very entities discovering the costly new therapies for rare disease. If the adoption of ICER-style reviews were to become widespread, it would likely have a harmful economic impact on New England, as these reviews would target the very therapies being developed here. The impact upon the industry could be significant. A recent [Deloitte report](#) pointed out that projected 2018 returns on research and development investments by the biopharma industry are the lowest since Deloitte began tracking this data. Widespread adoption of the ICER model in the US is quite likely to accelerate some of the adverse trends in the biopharma industry.

At the very least, depending upon the conclusions of some of these ICER reviews, the adoption of ICER reviews is likely to steer companies away from research into certain therapeutic areas that ICER deems less cost effective. Therefore, certain groups of patients would likely see less innovation toward cures for their particular diseases.

To inform this debate, Pioneer Institute will be conducting research during 2019 on the ICER methodology and its potential impact, most importantly the impact upon certain vulnerable groups of patients, but also upon the regional economy. In the meantime, Pioneer has prepared a list of questions policy makers may choose to ask

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when considering the adoption of ICER-style cost effectiveness reviews. The questions are not designed to offer definitive

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conclusions about ICER, but they represent a summary of the questions and concerns raised in academic literature about the limitations of the ICER model. We are hopeful that the questions themselves may illuminate some of the potential impacts of the

ICER model on patients, physicians, and drug discovery and, in this way, help policy makers reach the most thoughtful conclusions about use of the ICER methodology.

## Questions about the Potential Limitations of the ICER Model

In 2016, the *Journal of Stem Cell Research & Therapy* conducted a literature review of the limitations of the application of the Quality Adjusted Life Year methodology to cost effectiveness reviews; as discussed, QALY is the core measuring stick of the ICER model. The article argued that the academic literature displayed three general categories of limitations in the use of QALY:

1. Ethical Considerations
2. Methodological Issues and Theoretical Assumptions
3. Contextual or Condition-Specific Considerations

These three categories seem a useful way to organize questions that legislators or policy makers may have about the ICER model. First, is it ethical? Does the model discriminate against certain therapies needed by vulnerable patients and do those restrictions cross an ethical line beyond mere fiscal prudence and into an ethically dubious realm that might be described as rationing and anti-life?

Second, does the methodology used by ICER involve flawed “[measurement techniques, tools, assumptions and mathematical operations](#)” that call into question the validity of the model?

Finally, because ICER effectively uses the same model to analyze most therapeutic areas, is it a reliable guide to assessing the value of different therapies across disease categories? It is widely recognized that the challenges, circumstances, genetic variations, and quality of life issues vary greatly among different disease states, so it must be asked whether a single model can capture and analyze this complexity and apply it compassionately to a variety of patient circumstances.

## Ethical Considerations

Since the Hippocratic Oath of the fifth century BC, most important civilizations have affirmed that the provision of medical care has a strong ethical dimension and that there are certain ethical standards that must be upheld. Without making judgments about the ethical validity of the ICER model, it is certainly conceivable that a cost effectiveness model that undervalued human life could represent an ethically dubious approach to medical care. Here are some questions from the academic literature that may assist legislators in forming an opinion on the ethical component of ICER:

1. Is it ethical to deny patients a new therapy pending an ICER review?

Comment: The political controversy in the UK surrounding oncology drugs was related to the National Health Service’s unwillingness to provide a new renal cancer drug until the NICE review was complete. Some observers believe it would be unethical to deny a treatment, particularly a life-saving or life-altering treatment, pending a cost effectiveness review.

2. Do QALY-based reviews capture the real-world experiences of patients with particular therapies?

Comment: Reviews based upon QALY standards do not generally capture patient reporting on their experiences with particular medications, an increasingly important data point for physicians, the FDA, and health plans. While ICER does “consult” with patient advocates, patient data is not incorporated into its reviews. Patient reporting seems a valuable data point that is omitted in most ICER reviews.

3. Do QALY standards discriminate against the disabled by assigning a lower quality of life score for disabilities?

Comment: Under the QALY standard, quality of the life for people with disabilities is valued lower than for those who do not live with disabilities. This is because most therapies will never restore a disabled person to perfect health so, by definition, the value of treatments for the disabled will be undervalued. A patient living in a wheelchair may rightly believe that his or her life should be assigned the equivalent value of someone who is not disabled.

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4. In a related question, does the QALY standard discriminate against older Americans by denying them palliative care?

Comment: Since the QALY standard rates therapies on the basis of longevity and quality of life, seniors at the end of their lives might be denied patient care as that care may neither extend life nor greatly increase quality of life. In short, since an older patient's life, by definition, can be extended less than that of a younger person, are the elderly less likely to gain access to treatments?

5. Is the use of ICER reviews simply a method of dodging political accountability for rationing medicine?

Comment: In 1995, UK Minister of Health Gerry Malone was asked to decide whether the National Health Service should pay for Beta-interferon for multiple sclerosis. He made a compromise decision to cover the drug in certain cases. But he later told his staff that he never wanted to be faced with such decisions and they needed to develop an alternative mechanism to make these decisions. Malone **told** his staff: "This is not something that in my view should ever again land on a minister's desk." Malone's desire to avoid accountability for these difficult decisions led to the creation of NICE. It might be asked if governments relying solely on ICER's reviews is simply a device used by politicians to avoid responsibility, and provide political cover, for denying drugs to patients under the guise of an "independent review"?

6. Wouldn't the use of ICER reviews drive profitability for private sector health plans and pharmacy benefit managers (PBMs), and represent a conflict of interest?

Comment: The founder of ICER as well as many of its staff and corporate members, such as health plans and PBMs, believe drug costs need to be limited. However, for health plans and PBMs, any drug cost reductions would likely take the form of higher rebate payments from drug manufacturers, increasing health plan and PBM profitability. Significant controversy has already been generated around the nation about the problem with using list prices and the conflicts of interests that this creates for payers and PBMs. Rebate payments and discounts to health plans and PBMs now top **\$100 billion**, a figure that would likely rise significantly were ICER reviews to instigate deeper rebate payments to conform to ICER's cost effectiveness recommendations. ICER's funding therefore raises questions about its independence and potential conflicts of interest. Will the use of ICER exacerbate a problem that CMS is trying to eliminate?

7. Doesn't the QALY standard simply place an arbitrary value upon human life?

Comment: While it is an accepted practice among some economists to assign a monetary value to human life, the ethical implications of such a technique may be perilous. This opens an entire set of questions that might better be settled by religious leaders, ethicists and physicians; questions such as: Is the monetary value of a small child or a young mother the same as an older and frail patient? Should economists be making these value judgments?

8. Does the ICER review process interfere with autonomous physician-patient relationships?

Comment: Physicians and patients currently make drug therapy decisions based not simply upon the efficacy of the drug options but also upon potential costs to the patient. An ICER review that precludes the availability of an expensive drug takes the decision out of the hands of the physician and patient.

9. Is employing the ICER model a form of generational discrimination?

Comment: The effect of negative ICER reviews in certain therapeutic classes will undoubtedly diminish the interest of biopharmaceutical companies in conducting research in those classes. Therefore, young patients, and patients yet to be born, who will contract these particular diseases are less likely to have a cure in the future.

## Methodological Issues and Theoretical Assumptions

10. Is the use of meta-analysis, i.e. the pooling of results from different studies with different assumptions and analyzing different targets, often using different methodologies, a sound way to reach conclusions about specific drug therapies?

Comment: Some ICER critics have argued that metadata analysis can be an unsound method for reaching accurate conclusions about specific drugs when the wrong studies are combined into a single study.

11. Are ICER reviews conducted with adequate data?

Comment: Typically, ICER conducts reviews shortly after, or even before, a therapy is approved by the FDA. This time frame limits the review's ability to gauge the efficacy of a therapy in a larger numbers of patients or over a longer timeframe. Many times, health plans reach valuable conclusions about the safety and efficacy of a medicine after it has been used by many thousands of patients over a long period of time. This larger and longer view may call into question the reliability of an earlier ICER review.

12. Does QALY analysis lead to inefficiencies in spending in the healthcare system?

Comment: Some economists would argue that because QALYs establish arbitrary limits on drug spending, resources are then diverted to other less deserving components of the health care system, creating inefficiencies.

13. Does QALY help legislators address budget challenges and shortfalls?

Comment: QALY is intended to be an independent evaluation of therapies based upon their value under the QALY assignment of monetary value to a therapy. The QALY determination is wholly unrelated to the size of budgetary challenges of an individual state or payer.

14. Is ICER methodology overly quantitative and does it therefore fail to capture the variety of diverse circumstances that medical care presents?

Comment: The delivery of medical care is a highly complex undertaking informed by a variety of factors related to age, gender, mental health, cost, etc. Attempting to capture all these factors in a single quantitative model may be problematic.

15. Should quality of life measurements be determined by patients or the general population?

Comment: QALY standards generally value quality of life improvement by consulting with the general population. Some patient advocates have argued that, for example, improvements in quality of life for oncology treatments should be measured by consulting with cancer patients who are more acutely aware of the value of treatments. Shouldn't ICER reviews collect data points from both the general public and specific patient populations when conducting their reviews? One [study](#) seems to indicate that patient input is not adequately represented in ICER reviews.

The delivery of medical care is a highly complex undertaking informed by a variety of factors related to age, gender, mental health, cost, etc.

16. Should the ICER methodology be transparent?

Comment: Not all aspects of the ICER model are publicly available. When conducting reviews for public programs, some observers believe the ICER model should be completely transparent so it can be evaluated in the public square.

17. How often should ICER reviews be updated?

Comment: Some health policy experts argue that isolated reviews of a single drug therapy will soon become obsolete

because issues such as when improvements in one drug therapy may improve the effectiveness of another therapy make standards of care an ever-evolving target. For example, improvements in the treatment of diabetes may improve the effectiveness of treatments for heart failure. How will ICER capture these evolutions in the standard of care?

18. Is the use of list prices in ICER reviews a serious methodological flaw?

Comment: There has been exponential [growth](#) in the size of rebates, discounts and other fees paid to health plans and PBMs by drug manufacturers. By failing to capture these discounts, the ICER methodology doesn't accurately capture drug prices and ICER cost effectiveness reviews may involve errors of 30 percent or more for specific drugs, depending upon rebate levels.

19. How long will ICER reviews take and will new drugs be available to patients pending the reviews?

Comment: In the UK, NICE reviews for oncology drugs took so long that Parliament circumvented the NICE process and established a fund for cancer treatments that ignored NICE reviews. Patient advocates are concerned that ICER reviews will exhibit similar limitations in the US.

20. Is it arbitrary to establish a global budget for drug spending?

Comment: The ICER model caps annual drug spending. Ignoring the ebb and flow of biopharmaceutical pipelines may result in arbitrarily denying patients numerous new treatments in a year when the pipeline is particularly robust.

21. Does the utilization of QALYs fail to capture the non-health benefits of drug therapies?

Comment: Restoring a patient to good health can bring a variety of economic benefits not captured in the ICER model such as economic productivity, return to caregiver status, better performance in school, etc. The ICER model fails to adequately capture these types of economic benefits.

22. Do QALYs discount the opinion of physicians in patient care?

Comment: The decision to prescribe a particular drug to a particular patient cannot be made on the basis of a meta-data study, as only a patient's physician can understand the complexity of the individual patient's circumstances.

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### 23. Does the ICER model discourage innovation?

Comment: The US legal and regulatory regime for medicines encourages innovations in drug therapies that tend to make them expensive when they are patent-protected but inexpensive when the patents lapse. Because ICER only evaluates therapies based upon the patent-protected price, their model fails to capture the societal benefits of new medicines once they become far less expensive.

## Contextual or Condition-Specific Considerations

### 24. Is the ICER model inadequate to evaluate orphan drugs and drugs for rare diseases such as gene therapies?

Comment: ICER has developed an alternative methodology to evaluate therapies for rare diseases. However, rare disease clinical trials will, by definition, involve smaller number of patients and less robust data. For rare diseases, real world experience may provide sounder conclusions than an ICER review. Moreover, public opinion surveys indicate that the American public is willing to devote greater resources toward patients with rare and difficult diseases, a value judgment that may not be adequately captured in the ICER model.

### 25. Does the ICER model discriminate against preventative medicine?

Comment: The ICER model assigns value based upon improvements in quality of life and longevity. The value of medications that prevent disease do not seem to be adequately captured by this model.

### 26. Will personalized medicine make the ICER model obsolete?

Comment: Medical care is moving away from the “one-size-fits-all” model and toward precision medicine based upon breakthroughs in genetics. Through diagnostic testing, physicians will know which patients will respond to a certain therapy and which will not. ICER reviews may fail to adequately capture efficacy and side effect variations based upon a patient’s genetic profile.

### 27. Can the ICER model adequately capture the value of mental health treatments?

Of all disease categories, mental health is the most difficult therapeutic area in which to capture the value of various treatments to individual patients. There are some [data](#) to suggest that general public opinion, which ICER uses to weight quality of life (HRQoL) measures, underestimates

the impact of mental health conditions compared with the opinion of patients.

### 28. Does the ICER model, like the NICE model, have an inherent bias against cancer treatments?

Comment: As discussed, the greatest controversy around NICE developed from their reviews of oncology treatments. Because the ICER model favors treatments on the basis of longevity, it may not properly value an oncology treatment that, for example, may extend a patient’s life by three months over current therapies. The model’s bias may be particularly acute against treatments for very aggressive cancers.

### 29. Does the use of QALYs fail to capture the value of important nuances within specific disease areas?

Comment: For the treatment of rheumatoid arthritis, for example, the patient’s quality of life—not longevity—is the only meaningful measure of value. For a cancer patient, longevity is, by far, the most important value. The ICER model utilizes the same model regardless of differences in patient values by disease area.

## Conclusion

Trends in biopharmaceutical research have pushed reimbursement for very expensive medications to the forefront of public policy debates. Policy makers must make difficult choices between costs and patient access to these new treatments.

ICER provides policy makers with one potential methodology—some would say a flawed methodology—to evaluate the cost effectiveness of treatments using a set of assumptions that have significant limitations.

Because of these limitations, including ICER’s methodology and process, at this time our recommendation is for policy makers to avoid using ICER reviews for their Medicaid and other state programs. Whether a treatment is included in a formulary is a life and death decision.

We cannot recommend adoption of a tool that raises so many ethical, methodological, and disease-specific questions. The British experience with cancer care should be a warning for legislators to probe deeply about the implications of the ICER model for older Americans, the disabled, cancer patients and those with rare diseases. Until these issues are fully addressed, the ICER model should be avoided.

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## About the Author

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## About Pioneer

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