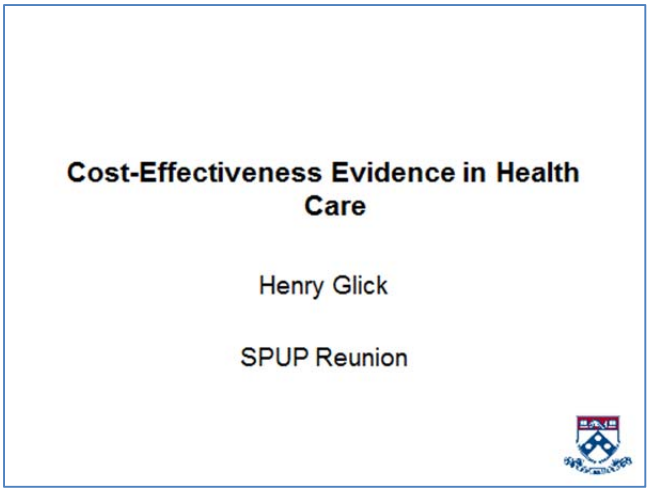
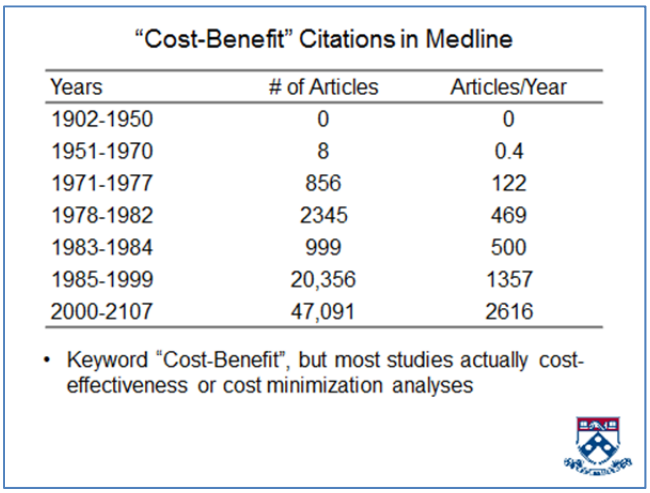


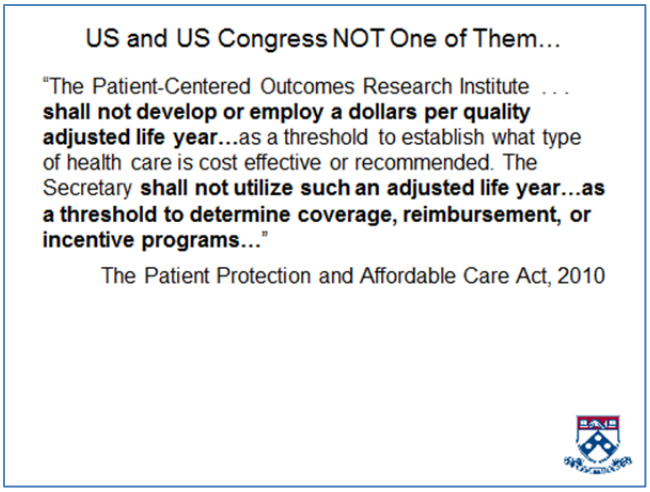
Good afternoon,

My research focuses primarily on cost-effectiveness analysis of health care, particularly methods development but also applied evaluations of clinical and behavioral health interventions



There were very few publications in this field before we arrived at SPUP. There were the beginnings of a literature while we were there. But as you can see in this table, the number of articles began to explode after 1985.

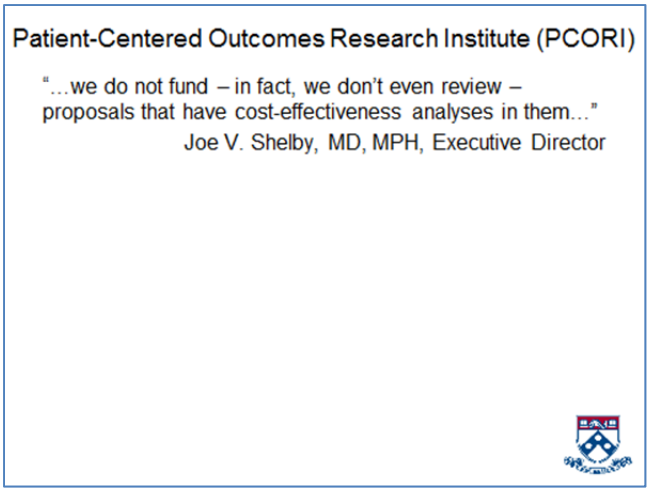
Internationally this explosion has supported widespread use of cost-effectiveness analysis in health care decision making. Probably the most important international institute is the UK National Institute for Health and Care Excellence or NICE (which its detractors sometimes refer to as NASTY: Not Available, So Treat Yourself). It's evaluated hundreds of therapies and is looked to by many other countries for methods and recommendations.



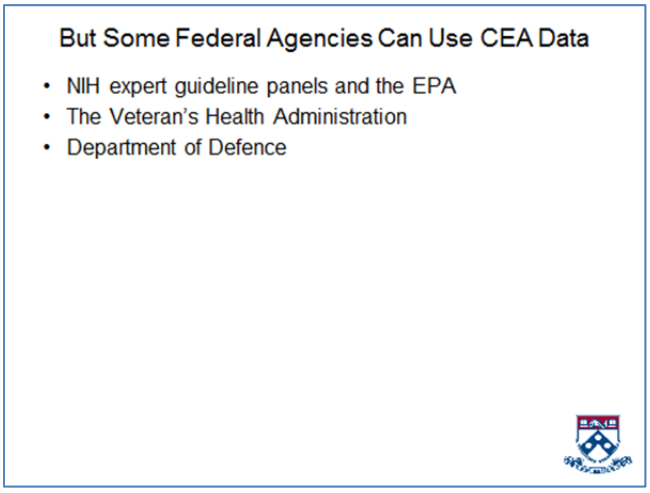
The US led by our Congress is NOT one of those countries. In fact even before the current administration, the US has a long history of legal prohibitions on the use of cost and cost-effectiveness data when making health care decisions. One provision of the Affordable Care Act states that "The Patient-Centered Outcomes Research Institute … **shall not develop or employ a dollars per quality adjusted life year …** as a threshold to establish what type of health care is cost effective or recommended. The Secretary **shall not utilize such an adjusted life year … as a threshold to determine coverage, reimbursement, or incentive programs …**"

By law, the Centers for Medicare and Medicaid Services can't use cost or cost-effectiveness in determining coverage policy or payment rates. They generally don't cover new therapies if they are less effective or are no better and cost more than other therapies. If the new therapy is definitely better, they generally cover it no matter what it costs. If there is a role for economic data, it usually is to trigger a closer look at the effectiveness data.

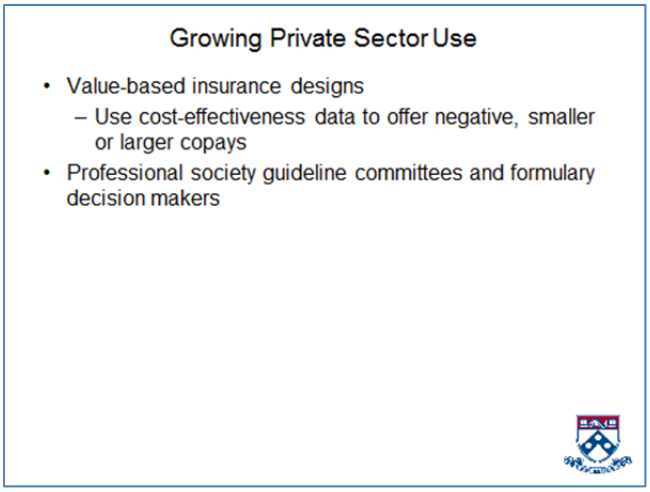
Similarly, while the NIH is a big funder of economic evaluation of health care, every so often a bill is introduced in Congress to forbid such funding, and the Food and Drug Administration can't consider cost in drug and device approval decisions



Finally, the patient-Centered Outcomes Research Institute or PCORI is an independent, not-for-profit corporation established by the Affordable Care Act to be a leader of comparative effectiveness research. Although not strictly required to do so by law, its Executive Director Joe Selby has stated publicly that "…we do not fund – in fact, we don't even review – proposals that have cost-effectiveness analyses in them…"

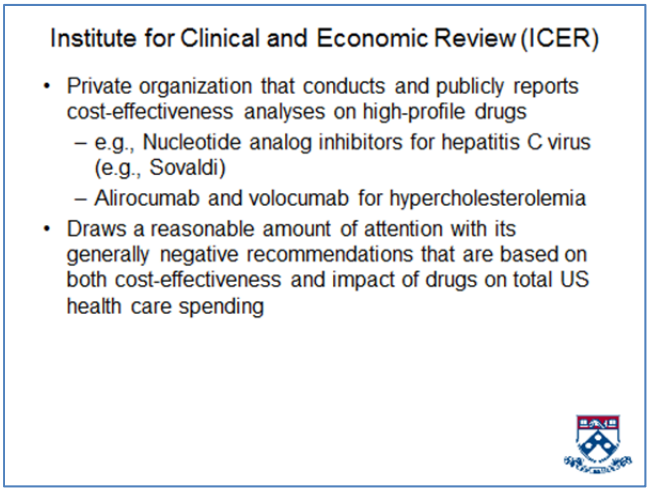


But some Federal agencies are allowed to and do use data from cost-effectiveness analysis. For example, the National Institutes of Health expert guideline panels and the EPA use such data. The Veteran's Health Administration and Department of Defense use them as well.

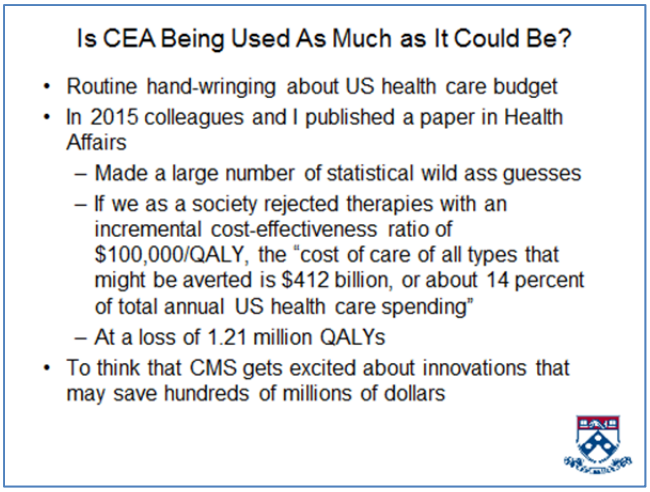


There is also growing use in the private sector. For example, there has been growth in the number of value-based insurance designs offered particularly in employer-sponsored health plans. They use cost-effectiveness data to determine if a medical therapy should have negative, smaller, or larger copays

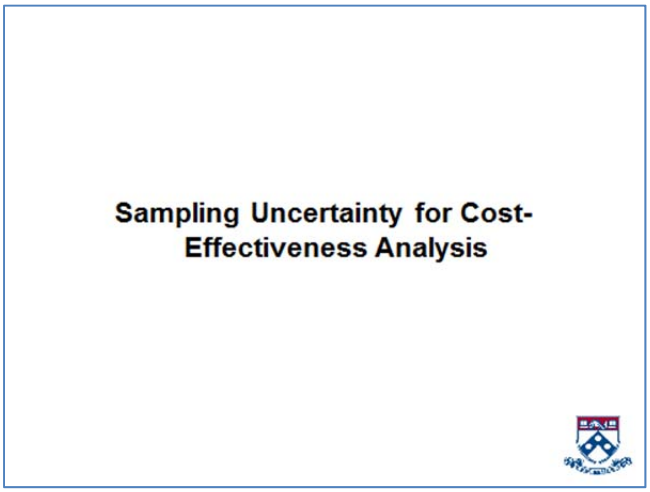
Similarly, professional society guideline committees and formulary decision makers often use or misuse results of cost-effectiveness analysis when coming up with guidelines or making coverage decisions.



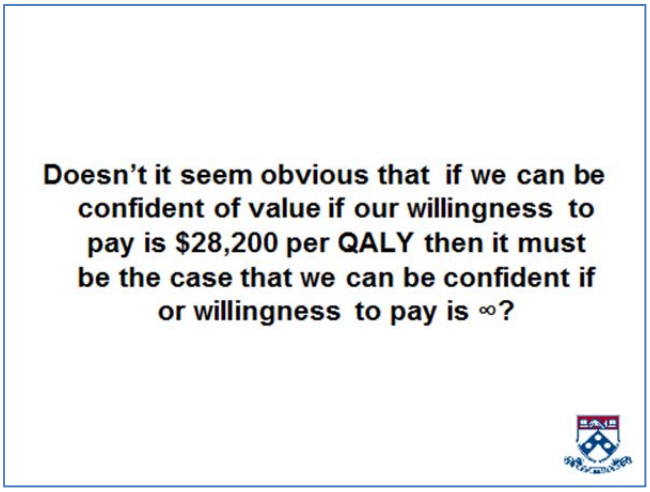
Finally, the Institute for Clinical and Economic Review or ICER is a private organization that conducts and publicly reports cost-effectiveness analyses for high-profile drugs. For example, it has looked at the $84,000 per course nucleotide analog inhibitors for hepatitis C and the $14,000 a month biologics for hypercholesterolemia. It draws a reasonable amount of attention with its generally negative recommendations that are based on both more and less well done cost-effectiveness analyses and their estimate of the impact of the drugs on total US health care spending.



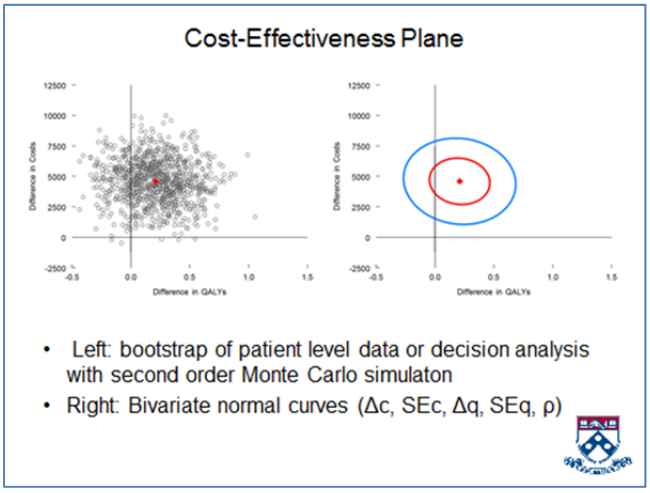
There is routine hand-wringing about the US health care budget. In 2015 colleagues and I published a paper in Health Affairs that looked to quantify potential savings if we rejected therapies whose cost-effectiveness ratios exceeded $100,000 per QALY. After making a large number of statistical wild ass guesses we reported that if as a society we rejected these therapies, the "cost of care of all types that might be averted is $412 billion, or about 14 percent of total annual US health care spending…." This savings would come at the expense of 1.21 million QALYs lost, for a savings of $340,000 per QALY lost. To think that CMS gets excited about innovations that may save hundreds of millions of dollars.



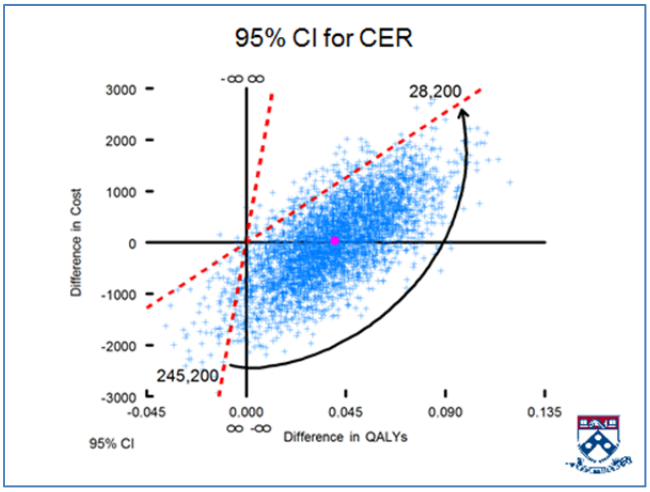
Since we are talking about evidence and its use or lack thereof, I want to close with a short discussion of measures of sampling uncertainty for cost-effectiveness analysis, which are one of my areas of interest. When we were at SPUP methods for evaluating this uncertainty were unknown. At the time, we didn't evaluate confidence intervals for cost-effectiveness ratios, we did sensitivity analysis. The first paper to propose methods for developing such intervals was published in Medical Care in 1994.



Confidence intervals for cost-effectiveness ratios can be particularly confusing. For example, doesn't it seem obvious that if we can be confident of value if our willingness to pay is $28,200 per QALY then it must be the case that we can be confident if or willingness to pay is ∞?



Sampling uncertainty is typically depicted on the cost-effectiveness plane. The X-axis represents the difference in QALYs, the Y-axis represents the difference in costs. As shown, we can display the bivariate distribution of these differences as either a scatter plot if our data come from a bootstrap or a second order Monte Carlo simulation or as a series of ellipses if our data can be reasonably thought to be distributed bivariate normal.



Here is a graph that represents an experiment in which we can be confident of value if our willingness to pay is 28,200, but not if it is ∞. It depicts an experiment in which the difference in cost is not significant because there is too much density above and below the X axis and the difference in effect is not significant because too much density falls to the left and right of the Y axis. Yet we can be 95% confident of value if we are willing to pay 28,200.

Counterintuitively, in this experiment the lower limit is 245,000; the upper limit is 28,200; and the point estimate is 875. The confidence interval ranges from minus infinity to 28,200 and from 245,200 to infinity and for these values of willingness to pay we cannot be confident . Values of willingness to pay between 28,200 and 245,200 fall outside the interval. Thus, if our willingness to pay is 50,000, or 100,000, or 150,000, we can be 95% confident of value. Even though the relationship between the limits and the point estimate may be confusing and even though neither the cost nor QALY differences are significant, the policy recommendations from this experiment are straightforward

Finally, as a last partisan comment about cost-effectiveness analysis and health care policy, one conclusion I have reached from my research is that we will not solve the problems with the health care budget by providing more preventive services or ensuring greater use of electronic medical records. These problems will continue until we as a society recognize that there are therapies that provide too little additional benefit for their costs, and are willing to reject their adoption.

Thank you