In 2008, an Oregon woman dying of lung cancer was denied coverage for Tarceva, a drug costing $4,000 a month. She received health insurance through the Oregon Health Plan (OHP), the state’s Medicaid plan, which in the early 1990s had made radical changes to its coverage decisions in an effort to increase the number of enrollees while also curbing spending growth. One of the most controversial measures was a list of 668 medical procedures, ranked according to their cost-effectiveness; the OHP would cover only the first 568. Tarceva, which extended life by a few months for a small percentage of patients, didn’t make the cut. (In response to the public outcry, the drug’s manufacturer, Genentech, provided the drug free of charge; the woman died a short time after starting it.)

Oregon’s list of treatments was based on cost-effectiveness analysis, a technique used to compare both the efficacy and cost of different medical treatments. The technique is politically controversial and methodologically challenging, but many health care experts believe it is a valuable tool for helping to allocate resources in the face of mounting health care spending.

Are We Spending Money Wisely?

Americans spend a lot of money on health care: $2.9 trillion in 2013 (the most recent year for which the Centers for Disease Control and Prevention has data), or 17.4 percent of GDP. That’s an increase from just 5 percent of GDP in 1960, and the Centers for Medicare and Medicaid Services (CMS) projects that health spending will continue to outpace GDP, reaching 19.6 percent of GDP by 2024. Rising spending reflects the rapid increase in health care costs, which have been well above overall inflation since the mid-1980s. Health care inflation slowed somewhat as a result of the 2007-2009 recession, but the CMS expects health care inflation to return nearly to pre-recession levels over the next five years.

Federal, state, and local governments provide a substantial portion of health care spending: Medicare, Medicaid, the Children’s Health Insurance Program, and insurance subsidies from the Affordable Care Act make up about one-quarter of the federal budget, or $836 billion. (About two-thirds of that money, $511 billion, went to Medicare.) In 2013, federal, state, and local governments paid for 43 percent of all national health spending, a share the CMS projects will rise to 47 percent by 2024.

The United States spends significantly more than other developed countries. In 2013, for example, the United States spent about $8,700 per capita on health care, compared with an average of about $3,900 for the other Group of Seven countries (Canada, France, Germany, Italy, Japan, and the United Kingdom). Growth in U.S. per capita spending also has outpaced growth in other countries. In part, the high level of spending reflects the United States’ relatively high per capita incomes; research has shown that health spending tends to increase with income. But in a 2008 report, researchers at the McKinsey Global Institute calculated that the United States spends about $2,000 more per capita than expected based on income levels.

High and increasing health care expenditures are not necessarily a cause for concern in and of themselves. “Increasing spending is usually a signal that the product or service is one that brings people more benefits than they could derive from spending the same amount of money on other available commodities,” says Henry Aaron, a senior fellow in economic studies at the Brookings Institution. “The issue with health care is that most of us don’t pay market prices, which can lead to the purchase of health care services where the value is less than the total cost of producing them. We may be consuming some services with only a slight marginal value.”

That view is borne out by multiple studies of Medicare data showing that regional variation in spending is uncorrelated with the quality of health care or with health outcomes. Patients in higher-spending areas see more specialists, get more tests, and spend more time in the hospital, but they aren’t healthier. Many researchers believe that the absence of a link between spending and outcomes reflects a high level of unnecessary care — as much as 30 percent of all health care costs, according to the authors of one Medicare study.

Many potential health care reforms, such as high-deductible insurance programs where consumers bear more
Calculating a “QALY”
In medical research, cost-effectiveness is a ratio that expresses health outcomes in terms of dollars spent. The numerator of the ratio is the cost of one unit of outcome and the denominator is the unit of outcome, such as the number of health states prevented by a vaccine or the number of new diagnoses made by a screening test. One widely used denominator is a Quality Adjusted Life Year, or QALY, which takes into account not only extending life, but also the quality of a person’s health during that life. (Technically, research using QALYs is a subset of cost-effectiveness analysis known as cost-utility analysis, but researchers generally use the broader term.)

A QALY is based on a number known as a “health utility,” which runs on a scale of 0 to 1, with 0 being death and 1 being perfect health. This utility value is then multiplied by a number of years. If a treatment increases health utility, extends life, or both, the number of QALYs increases. For example, Aaron Carroll and Stephen Downs of the Indiana University School of Medicine have estimated that mild intermittent asthma in children has an average utility value of .91 and severe seizure disorder in children has a much lower average utility value of .70. Thus, returning a child with asthma to perfect health for 60 years would gain 5.4 QALYs, and the child with the seizure disorder 18 QALYs.

In the view of researchers using this approach, such calculations enable doctors and policymakers to compare different health problems and their treatments. Hypothetically, if curing intermittent asthma and curing severe seizure disorder both cost $1 million, the cost-effectiveness would be about $185,000 per QALY for curing asthma and about $55,500 per QALY for curing severe seizure disorder, making it more cost-effective to cure the latter.

There are several different techniques for calculating health utilities. One is based on the “standard gamble,” which was developed by mathematician John von Neumann and economist Oskar Morgenstern in their 1944 book, Theory of Games and Economic Behavior. An individual is given a choice between a certain health state and a gamble that could lead to a better or worse outcome. The probability of the better outcome that would make them indifferent between their current state or taking the risk is the utility of their current state.

Another method is the “time trade-off,” in which individuals are asked how many years of life they would be willing to give up in order to live without a certain condition. For example, a recent study that used the time trade-off to calculate health utilities for epilepsy asked respondents to choose between living for 10 years with frequent seizures or living for X years in perfect health. They found a utility of .303, meaning respondents would prefer living for about three years in perfect health to living for 10 years with frequent seizures.

The standard gamble and time trade-off are both direct methods, where researchers ask people about specific diseases. But researchers might also use indirect methods, where people are given a simple questionnaire and asked to rank generic health states, such as living with reduced mobility or requiring assistance with daily tasks. Several indirect questionnaires are widely used by researchers. In general, they are developed by asking a sample of the public how they value a certain limited number of health states and then applying an algorithm to map those health states onto other conditions to derive utility values for a wide range of conditions.

In much of economics, utility is an ordinal value; a consumer might get more utility from buying oranges than from buying apples, but it’s not possible to actually measure how much more utility they get. Such ordinal utility values cannot be compared from person to person. In the QALY methodology, however, a health utility is a cardinal value; a utility of .08 is four times better than a utility of .02. As a result, it is mathematically possible for researchers to compare utilities across individuals and calculate an aggregate health utility for a given disease state.

Proceed with Caution
Health utilities can vary widely from study to study depending on the method used to calculate them and on the survey sample. For example, patients already living with a certain disease tend to place a higher utility value on that health state than respondents who are asked to imagine living with that disease. Or a young athlete might assign a much lower utility value to a torn ligament than an elderly person. In addition, the standard gamble generally results in higher utility values than the time trade-off. That’s because people tend to be risk averse and thus require a high probability of an improved outcome in order to take the gamble.

QALYs can also vary in context depending on how a certain technology is used. For example, as Weinstein noted in a 2005 lecture at Syracuse University, many people who have suffered a heart attack routinely receive an angiogram to check for blocked arteries. For patients who are at high risk of having a blocked artery, the procedure gains between 20 and 50 QALYs per $1 million. But for patients who are at low risk, the procedure gains less than 10 QALYs per $1 million.

It also can be difficult to determine how effective a treatment is because, as Weinstein says, “You can’t conduct a randomized controlled trial of every intervention, or with every potential category of patient.” For that reason, researchers have begun tapping into other data sources,
such as insurance claims and coordinated medical records, to establish an evidence base for evaluating effectiveness. And even treatments deemed to be effective might have lower-than-expected returns given the deleterious effects the treatments themselves can have on life quality.

Ethical questions also arise about whether different weights should be assigned to people of different initial health states or of different ages. For example, as Steven Pinkerton of the Medical College of Wisconsin and several co-authors noted in a 2002 article, people with substance abuse problems tend to be in worse health on average, so a given intervention might bring them to a health state with a lower utility value than the same intervention would for a person in better health. But by that logic, substance abusers would be less deserving of health care. And, Weinstein asks, “Should we assign more weight to people at the end of life because their remaining years are precious? Or should we assign more value early in life, because once a person has reached a certain age they’ve already had an opportunity to live a healthy life?”

Some researchers have argued that these methodological questions render the QALY useless as a metric. But many health care experts believe that while QALYs should be interpreted with caution, they are a valid tool. “Decisions about resource allocation are being made all the time,” says Weinstein. “We can make them on an ad hoc basis, or we can make them with the benefit of some sensible analysis about the benefits and harms.”

Cost-Effectiveness in Practice
Many industrialized countries use cost-effectiveness research to make coverage and reimbursement decisions for their national health insurance plans. In the United Kingdom, for example, the National Institute for Health and Care Excellence (NICE) generally recommends that treatments be covered by the National Health Service beneath a threshold of between £20,000–£30,000 ($30,300–$45,500) per QALY. (NICE’s threshold has been the source of considerable controversy, particularly with respect to expensive treatments for rare or terminal illnesses.) Other countries do not define a threshold as explicitly as the United Kingdom, although they do have implicit thresholds that inform coverage decisions. The World Health Organization’s rule of thumb is that one to three times GDP per capita is cost-effective, which in the United States would be between roughly $55,000 and $164,000.

But in the United States, cost-effectiveness prompts fears of rationing and “death panels” that would deny access to lifesaving treatment. In 1989, Medicare proposed using cost-effectiveness as one of several criteria, but the proposal met with significant opposition and was never adopted. In 2010, the Patient Protection and Affordable Care Act (ACA) created the Patient-Centered Outcomes Research Institute (PCORI) to conduct comparative effectiveness research, a method of conducting direct comparisons of different medical treatments that does not take into account cost. In establishing PCORI, Congress prohibited the institute from funding any research that considers cost at all and barred Medicare and Medicaid from considering cost-effectiveness as well. (The one exception is the Oregon Health Plan, which received special federal approval in 1993 for reforms including a treatment list based on cost-effectiveness and continues to use a prioritized list of treatments when determining coverage.)

Aaron notes that although the government monetizes life in a variety of circumstances, such as when it decides whether the “cost per life saved” justifies mandating a new safety standard for automobiles, people tend to find the idea unsettling. “When one monetizes the value of medical services, one is placing a value on either the extension of life or on improvements in the quality of life. And that’s something that a lot of people are very loath to do,” says Aaron.

Cost-effectiveness is widely accepted in the academic medical community; leading journals regularly publish studies on CEA, to the tune of 67 published studies in 2013, according to data from the Center for the Evaluation of Value and Risk in Health, a nonprofit research group. And among practitioners, says Weinstein, “there is considerably more acceptance of the need to consider cost and the limitations on resources when making recommendations for clinical practice.”

In 2007, the American Medical Association endorsed “value-based decisionmaking” as a strategy to achieve better value for the amount of spending, and specifically mentioned cost-effectiveness research as “essential” to provide doctors and patients with the information they need to make value-based decisions. In 2014, the American College of Cardiology recommended the use of cost-effectiveness analysis as one consideration in treatment guidelines, noting that “Despite [methodological] challenges … the need for greater transparency and utility in addressing resource issues has become acute enough that the time has come to include cost-effectiveness/value assessments and recommendations in practice guidelines and performance measures.” The American Society of Clinical Oncology followed suit with a similar statement in 2015, although it noted that considerable research remains to be done.

Private insurers also may include cost-effectiveness as one of several factors in deciding what to cover. The clinical policy at Aetna, for example, the third-largest insurer by market value in the United States, states that “when effectiveness and safety are equivalent, we may consider the cost-effectiveness among therapies to determine medical necessity or to require certain therapies to be tried before covering equivalent, but more expensive options.” Still, overall, cost-effectiveness plays a limited role in the United States health care market.

Does Cost-Effectiveness Work?
Given the complexity of medical care and of the health care market, it’s difficult to determine how much health outcomes might improve, or how much money might be
saved, if cost-effectiveness were more widely considered by insurers and practitioners. There are trade-offs with respect to health outcomes. As Weinstein and Jonathan Skinner, an economist at Dartmouth College, noted in a 2010 article in the New England Journal of Medicine, some treatments for late-stage pancreatic cancer might be considered cost-ineffective, while diabetes treatment is very cost-effective. Reallocating resources from one to the other might improve aggregate health outcomes, but it wouldn’t improve outcomes for patients with late-stage pancreatic cancer.

Research suggests the spending benefits could be large. In a 2009 New England Journal of Medicine article, Elliot Fisher and Julie Bynum of the Geisel School of Medicine at Dartmouth College and Jonathan Skinner found significant regional differences in the growth of Medicare spending, even after controlling for differences in health outcomes. Between 1992 and 2006, for example, spending rose 2.4 percent in San Francisco versus 4 percent in East Long Island. Over the course of the study, that difference accounted for more than $1 billion in extra Medicare spending just from East Long Island. If 30 percent of that spending could be cut without worsening health care quality, as other research has found, considering cost-effectiveness could help slow spending growth. Fisher and his co-authors estimated that reducing overall annual growth in per capita spending from the national average of 3.5 percent to the rate in San Francisco could save Medicare $1.42 trillion.

At the same time, however, research suggests that the Oregon Health Plan, the one real example of explicitly using cost-effectiveness data in the United States, did not succeed in reducing expenditures. An analysis by the Cascade Policy Institute, a nonpartisan libertarian research group, found that growth in Oregon’s Medicaid expenditures closely tracked the growth across the United States. In addition, the ultimate benefit of any savings resulting from cost-effectiveness analysis depends on how, or if, those dollars are reallocated to more cost-effective treatments or to other higher-value uses in the public or private sector.

Still, the potential is there, and as spending continues to rise, it will become more important to ensure that the money is being put to its best use — and that likely means paying attention to costs.

Readings


Richmond Fed Research Digest is an annual publication that brings the externally published work of the Bank’s research department economists together in one place. It includes brief summaries, full citations, and links to the original work.

Visit the digest at www.richmondfed.org/publications/research/research_digest