Addressing the Myths About ICER and Value Assessment

To get innovation and better access to new drugs we need prices that match the added value for patients, along with coverage policies that are fair for patients, insurers, health systems, and innovators.

We all want access to treatments that can help extend and improve our lives.

The good news is that innovative companies are producing a growing number of new drugs, many of which offer remarkable advances for patients with life-threatening conditions. But the price tag attached to these new drugs is often crushing. It's not hard to see that if most new treatments for a wide range of serious illnesses cost more than $100,000 per year, even when new treatments are only slightly better than existing options, the system will break. Many people think it already is broken. The status quo isn't good for anyone.

- Millions of patients, even those with good health insurance, struggle to afford their prescriptions, and many find that insurance coverage makes it hard to get access to new drugs.
- Health insurers ultimately raise insurance premiums to pay for the lion's share of higher drug costs, making health insurance less and less affordable for everyone.
- Faced with escalating insurance costs, some employers want to throw in the towel and stop providing drug coverage entirely, while Medicare and state budgets for Medicaid are under severe pressure.

How can we make sure that we can afford the innovation we want for patients in the future?

Ultimately, we need innovation *and* better access to care, and to get it we need prices that match the added value for patients, along with coverage policies that are fair for patients, insurers, health systems, and innovators.

Working to address this challenge has been the primary mission of ICER since our founding. ICER started in 2006 as an academic research group at Harvard Medical School with faculty blending expertise in health care ethics and evidence-based medicine. In 2013, ICER became an independent non-profit research institute. ICER has no statutory or regulatory power to make coverage or payment decisions. Instead, we fill a gap in our country's health care system by producing independent, scientifically rigorous reports that help support discussions of how to achieve the broader goal of improving patient outcomes while making health care more affordable for patients now and in the future.

To accomplish this goal ICER:

Works collaboratively with patient groups, clinical experts, and life science companies to develop reports comparing how well different treatment options work for different kinds of patients and analyzing how much each option costs the entire health care system over both the short and the long term. For new treatment options, we often calculate "value-based price benchmarks" based on evidence of how much better they actually are at improving patients' lives. The intent of our reports is to provide all participants in the health care system with a fair and objective view of the evidence that compares different treatment options. Through our pricing analyses, we also seek to promote a "win-win-win" outcome: a price that insurers will recognize as aligned with value; a price that would lead to broad and affordable coverage for all patients who can benefit; and a price that would fairly reward innovators for the value they bring to patients, and provide them ample incentive to pursue the investments and research that will lead to the innovative treatments of tomorrow.

Patient input has been a crucial part of our methodology from the beginning.

Convenes public meetings across the country to bring the public fully into consideration of questions of value. At these meetings, independent regional panels of clinical, scientific, and health policy experts discuss ICER's reports. At each meeting these independent panels hear the testimony of patients and others, following which they take votes on the strength of evidence behind different treatment options. Then, with input from representatives of the patient, provider, payer, and manufacturer communities, each meeting concludes with a policy roundtable that provides an opportunity for all stakeholders to share ideas on how the evidence might best be used to support clinical practice, pricing negotiations, and coverage policies that will improve patient outcomes and value.

Asking the Tough Questions

Any independent effort to analyze the evidence on health care treatments will create tension. Some new treatments will appear to be "winners" and others "losers"—always controversial when the short-term interests of individual patients, manufacturers, and insurers may be at odds with each other. Any effort that also seeks to analyze the value of new treatment options and suggest alternative pricing models will bring to the forefront difficult issues about money, power, and fairness in American health care, raising tough questions such as:

- Who profits and how much?
- Can we eliminate enough waste in the health care system to pay for new innovation?
- What is a fair price for a new treatment if only one company makes it and can charge any price it wants?
- How much is it fair to ask patients to pay themselves for expensive treatments?
- Do we ever decide that something is too expensive for the added value it provides individual patients?

ICER does not pretend to provide the answers to these questions. But we do profess a strong belief that our nation can do a better job of serving the patients of today and those of the future by looking objectively at the evidence, embracing the difficulty of comparing the value of different treatment options, and coming together in a public space to have an honest, civil discourse about the options for how to use evidence as the cornerstone of a more effective and efficient health care system.

The bottom line: everyone needs a price that makes sense. And patients should be at the center of that discussion.

Debunking Myths

As ICER's work has become better known and mentioned by insurers and others as an element in their decision-making, there have been questions about our intentions, methods, and the ultimate impact of our efforts. Some of the concern has been principled and thoughtful, reflecting different views on procedural and methodological aspects of our reports. We have always welcomed this input and have made numerous changes over the years to address the issues raised.

Unfortunately, much of the more recent criticism has been fueled by a lack of knowledge about ICER or even willful mischaracterization by those who oppose a move toward pricing in alignment with the added value for patients. Some of these distortions have taken on a life of their own, threatening to make it more difficult for patient groups and others to engage constructively in our report development and in the broader debates now going on about value assessment and drug pricing. We firmly believe we cannot do our work without the insights we gain from patients and patient groups. Therefore, as we continue to welcome honest criticism from all perspectives, we feel that it is important to set the record straight in the sections below on the most common and pernicious myths about ICER and our methods of value assessment.

For those involved in the effort to help patients, and those interested in the future of our health care system, we hope you will read further and engage with us to address any concerns you might still have.

Myth #1

ICER was founded by the health insurance industry, is largely funded by insurers, and continues to be a front for their interests with the primary goal being to cut costs and boost insurer profits.

Truth

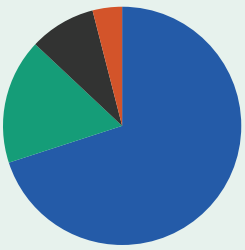
ICER was founded in late 2006 as an academic research project at Harvard Medical School, moved to the Massachusetts General Hospital in 2007, and became an independent non-profit organization in 2013.

In its first three years, the largest source of funding for ICER came from unrestricted support from the National Pharmaceutical Council. Overall, during its early years, funding for ICER came from the National Pharmaceutical Council (~40%), grants from the federal government (~25%), grants from non-profit foundations such as the Robert Wood Johnson Foundation and the Blue Shield of California Foundation (~25%), and unrestricted support from health insurers (10%).

Today, ICER receives 70% of its funding from non-profit philanthropic foundations, the largest source being the Laura and John Arnold Foundation, and no funder influences our research findings or even what drugs we choose to evaluate. Non-profit foundation support is used to support all of ICER's reports and public meetings. **For an annual Policy Summit meeting and evidence policy webinar program, ICER receives funding equivalent to 17% of overall support from pharmaceutical companies and only 9% from health insurers**. Support from state contracts contributes the remaining 5% of overall funding. Information on funding sources for ICER are updated annually and displayed on our website so that all stakeholders can have confidence in the impartiality of our work.

We do not represent the interests of the insurance industry. Our reports follow the evidence: some have found that the evidence on the comparative effectiveness of a new drug is extremely limited; for other drugs our reports have judged the evidence to be robust and persuasive. **Most of our reports have found that the list price of a new drug is too high for the amount of improvement it offers over other options, but some reports have found that the list price of new drugs can be well aligned with the added value for patients**. For example, one report concluded that the new drug Entresto™ for heart failure was so effective over the long term that its list price could be 2-3 times higher(!) than its actual list price, and proposed that the company should be commended for making the price even more affordable to health insurers and patients in the short term. We have also found that some new drugs, like those to treat opioid addiction, benefit patients and society, save money overall in the health system, and are outstanding values. Our aim is not to support one side in a negotiation; it is to provide what our health care system has lacked for so long: an independent, trustworthy source of information that can help serve all voices in the discussion on value.

Sources of Funding: 2016



- **70%** Non-profit foundations
- **17%** Manufacturer grants, contracts and contributions
- **9%** Contributions from health plans and provider groups
- **4%** Government grants and contracts

Myth #2

ICER ignores the patient perspective in its approach to value assessment and does not engage meaningfully with patient groups during the development of its reports.

Truth

Patient input has been a crucial part of our methodology from the beginning.

The ICER value framework was developed to facilitate discussions about value at the broader population level where pricing and coverage decisions are made, not to try to supplant the personalized decision-making that is critical for high quality patient care. But even though the ICER value framework is applied at the population level by insurers, provider groups, and drug makers, its structure reflects our belief that the assessment of value for all decision-makers should be grounded in what matters most to patients. The framework, which is available on the ICER website along with an hour-long webcast describing it, is grounded in the patient experience through several mechanisms:

1. **The framework anchors judgments of value in the evidence on comparative clinical effectiveness of treatment options, and explicitly acknowledges that patient guidance on the outcomes that matter most is critical to the assessment of value**. The value framework also specifies the importance of learning from patients and clinicians about possible patient subgroups for which the balance of benefits and side effects might be very different, requiring separate consideration in any value assessment. For example, in our report on PCSK9 inhibitors for high cholesterol, we divided our analysis into three sections to mirror the three different patient populations that patient groups and clinical experts told us should be looked at as distinct, and we found differing levels of evidence to support the use of these new medicines in these three groups.
2. **The ICER framework also includes categories called "additional benefits" and "contextual considerations" that are meant to be able to capture elements of value of importance to patients and their families that might not be included in the "clinical" literature**. Issues such as whether patients have had any other reasonable treatment options in the past, sites of care, ease of administration, impact on work and family life—all are given a formal place in the ICER framework and our reports have sections filled with the insights (and data when possible) provided directly by patients and patient groups. For example, in one recent report we highlighted the importance of an additional benefit we learned about from discussions with multiple myeloma patient groups: the benefit of having an oral medication option that could help many patients and families not make a long journey in to a clinical center for an IV treatment.
3. **From the very first day of our assessment process, which is over 7 months long, we talk with patients and patient groups and offer multiple chances for informal input as well as formal written and oral comment on draft versions of our reports**. We view patients as partners in our report process and are continuing to create new materials to help patient groups understand how they can help guide and inform our reports. Recently we have begun to seek opportunities to use evidence directly generated by patient groups, another key way in which we hope to make the patient experience and perspective central to our process.

Myth #3

The economic models that ICER uses as a key part of value assessment use the Quality-Adjusted Life Year (QALY) which discriminates against those with serious conditions and the disabled, "devaluing" their lives in a way that diminishes the importance of treatments to help them.

Truth

The QALY was developed by health economists and doctors in the United States and is now used throughout the world as the gold standard measure of how much better a treatment makes patients through extending life and/or improving the quality of life. Here's how the QALY works: if two drugs both help extend life for patients with cancer for an additional six months, but one drug has severe side effects that cause nerve damage, diarrhea, and blindness, we need a way to weight those harmful side effects so that our measure of the overall clinical benefit of the drug with fewer side effects is better than the other drug. That's what the QALY does. **The key point is that the QALY measures relative improvement from wherever patients start out. If the treatment is one to help patients who have a stroke achieve better functioning, the improvement in quality of life is not discounted in any way just because patients start out with a lower quality of life than someone in perfect health**. In fact, starting out with a lower quality of life, whether through a serious illness or disability, offers more "room" for improvement, giving treatments for patients with serious conditions more opportunity to show improvement compared to treatments for patients whose baseline condition is already near perfect health.

However, the surveys and other tools that have been developed to measure quality of life as part of the QALY often assign a lower quality of life to serious conditions. In many ways this is logical and appropriate because we want to capture the downside for patients of serious side effects, like nerve damage or blindness, which might be caused by some treatments. But this approach has the potential to create an ethical dilemma. For treatments that extend life but do not improve quality of life, the amount of credit (i.e. additional QALYs) attributed to a treatment will depend in part on how sick patients are given their underlying condition. For example, most people would agree that three more months of life in good health is better than three more months of life in a coma, but this means that less benefit, as measured by QALYs, could be attributed to treatments that extend life for patients with serious conditions than to treatments that produce the same extension of life for patients with less serious conditions.

ICER is aware of this possibility and we take several steps to address it. One way we minimize the potential effects is to select quality of life scores whenever possible from individuals who have the condition rather than asking people without the condition to judge "how bad" it would be to have that disease. People with serious conditions often rank their quality of life nearly as highly as individuals without the condition, so any concerns about the QALY unfairly penalizing certain treatments can be minimized by selecting quality of life scores from people with the condition being studied. Secondly, ICER repeats analyses using different quality of life assumptions in order to understand whether a change in baseline quality of life makes an important difference in the final results. These repeat analyses, called "sensitivity analyses," are important elements of our reports and are discussed with our independent panels when they consider the evidence at our public meetings. We remain sensitive to the implications for our results of the methods used to calculate the QALY, and we will continue to work with patient groups, academic experts, and others to evaluate and address perceived shortcomings.

Myth #4

ICER only cares about the short-term cost to insurers and uses an arbitrary budget cap to suggest low-ball prices.

Truth

ICER's value framework is anchored in the long-term perspective, with the cost-effectiveness of new drugs being evaluated using what is called the "lifetime horizon" for patients. The incremental cost-effectiveness ratio, also often called the "cost per QALY" that ICER economic models calculate, all use simulations of future years stretching out to the full lifetime of patients. This means, for example, that our calculations estimate and capture all the prevented heart attacks and hospitalizations that might be expected over more than 30 years for middle-aged patients receiving an effective new treatment for high cholesterol.

The myth about short-term costs and arbitrary budget caps has arisen because ICER's value framework includes two components: the long-term "care value" based on the lifetime horizon as described above; and a secondary consideration of the potential budget impact over the first five years following a new drug's introduction. Infrequently, a drug may represent an excellent value over the long-term but, because there are millions of patients potentially eligible to use the drug, spending to provide the drug for a sizeable part of the eligible population might strain health system budgets in the short term.

ICER therefore uses a transparent set of equations to look to see if the potential use of a new drug could pose such a short-term affordability problem, and our calculations are done explicitly to sound an "alarm bell" if the short-term costs might increase overall health spending significantly faster than the US national economy is growing. We have repeatedly and explicitly said that if the potential budget impact might exceed this "alarm bell" threshold it is not intended to function as a budget cap. It is simply to note that insurers, patient groups, drug makers, and other stakeholders may need to consider whether special measures should be put in place to help manage the possible short-term squeeze on health budgets. **Some drug makers have claimed that considerations of affordability have no role in judging the value of new drugs, but we continue to believe that short-term affordability concerns are often an important influence on insurance coverage and increases to insurance premiums, and that patients and all other health care participants should have the chance to discuss mechanisms that will ensure we can afford to provide good access to new drugs with excellent long-term value**.

Myth #5

ICER wants to take money from dying patients in order to "fix potholes."

Truth

This is one of the more remarkable and malicious mischaracterizations of our intentions.

In introducing the broader social and ethical questions that ICER reports are supposed to help address through public dialogue, we have shown data from the state of Massachusetts demonstrating that state spending on health care has risen nearly 60% in the last decade. We also show data on where that money came from: steep reductions in spending on every other major type of social service, including education, fire and police protection, housing, public health, and infrastructure. The same story is echoed in state houses around the country: health care costs are growing rapidly, severely impinging on the ability of states to maintain other services. This obviously does not imply that ICER wants dying patients to be denied treatment in order to fix potholes. What it does mean is that we hope that our reports and the public meetings we convene can lead to a more robust and honest discussion about the real choices and trade-offs that are being made in spending at the state and national level. **If the shared hope is to be able to provide innovative drugs for all patients with serious illness, and to be able to also afford good education for our children and other services, then we believe that transparent discussions about whether prices for drugs and other health care services are reasonably aligned with the value they bring to patients are an important way to help us get there**.

Myth #6

Insurers will always use ICER reports to restrict access for patients, often by making patients take less effective, older medications first.

Truth

Insurers have always made—and will continue to make—coverage decisions that restrict access to innovative new drugs for some patients. These decisions tend to happen behind closed doors without full transparency in the review of evidence or the justification of the coverage policies. ICER reports seek to provide a common, openly available and trustworthy resource for patients and all participants in health care discussions about coverage policy. Our public meetings seek to explore—from multiple perspectives—how the evidence can best be applied to clinical practice, and to pricing and coverage policies in a way that maximizes the benefits to patients while remaining sensitive to long-term value and affordability.

As part of the policy roundtable discussion at the ICER meeting immediately following the introduction of the first new drug for hepatitis C, clinical experts suggested that the most feasible way to manage the large number of infected patients needing treatment was to prioritize treatment for those with more advanced disease. Many insurers, whether they referenced the ICER report or not, adopted early policies covering new medications only in patients who had some evidence of liver damage.

On the other hand, ICER's meeting on new treatments for patients with multiple myeloma included a policy roundtable discussion in which major insurers noted that they did NOT feel that the nature of the condition and the available evidence supported the idea of patients being required to try less expensive drugs and "failing first" before being granted coverage for more expensive options. This policy perspective was included as one of the primary messages of the final ICER report.

It is true that, to date, most of ICER's reports have found that the list price of new drugs exceeds a reasonable threshold for the degree of improvement they provide over other treatment options. But does this mean that insurers should restrict coverage, that patients should just pay more out of pocket, or that the prices should be negotiated downward to align better with the added value for patients? **ICER's purpose is to stimulate a public discussion of these questions and we do not believe that the right answer is to restrict access to innovative medicines for patients who are likely to benefit. Patients already suffer restrictions to access when drug prices are too high for them to afford, and our goal is to provide a way to get to a "win-win-win" outcome where price is aligned with value, access is broad, clinical use targeted and appropriate, and new investments in future innovation assured**.

Myth #7

The long-term effect of ICER will be to stifle innovation and cut off research and development for the next generation of cures and other breakthrough therapies.

Truth

We do not want to stifle innovation. We want *better* innovation. Better innovation means innovation that is supported by evidence that helps patients and their clinicians understand the risks and benefits of different treatment options for individual patients. Better innovation means innovation that provides substantial benefits for patients and that is priced in accordance with how much it actually improves patient lives, not in accordance with what the market will bear. And better innovation is innovation that is priced and introduced into the health care system in a way that is more affordable for our health systems, our states, and our country.

Reasonable people will disagree on what amount of financial reward and certainty of that reward are required to maintain a healthy life science industry in the United States. The pharmaceutical sector has had the highest average profit margins within the overall stock market for decades, and its companies and CEOs consistently rank among the most successful in the US business landscape. We do not believe that aligning prices more reasonably with the added value for patients will cripple or even severely harm the underlying dynamic in our pharmaceutical industry that continues to develop new cures and other breakthrough therapies. In fact, we believe the opposite: that if prices do NOT come into alignment with value, a deeper crisis in patient access and societal affordability will occur, potentially creating a true threat to future innovation.

The bottom line: everyone needs a price that makes sense. And patients should be at the center of that discussion.