

Our Journey Towards a More Equitable U.S. Health System





Our Mission

ICER is dedicated to advancing the use of evidence to improve health care affordability and access for all patients and their families.

Putting Patients at the Center of a More Ethical Health Care System



Prior to forming the Institute for Clinical and Economic Review, as a primary care physician in various practice settings, I experienced firsthand how the U.S. health system fails patients. I saw parents with health insurance feel ashamed to ask for free samples when they couldn't afford treatments for their children. I saw my fellow doctors prescribing expensive drugs without thinking about the cost, and without really knowing anything about the evidence demonstrating that equally effective, or better, options were available at lower cost. And I saw laboratory tests abandoned, pills not taken, or rent not paid and insurance dropped – all because the cost of health care in the wealthiest nation on earth was unaffordable to many of its own people.

These experiences left me profoundly troubled. The dysfunction I was seeing radiated in all directions, affecting everyone in the health care system, but especially patients and people caring for them. The problems seemed so deeply embedded that I wasn't sure what could be done. But I started talking more openly about these problems with my own patients and with my colleagues, and traveled broadly to learn from people, companies, and governments trying to solve them. As I learned, a road forward started to emerge. It was clear that these challenges aren't due to any one single factor; not the lack of good evidence, or the failure to talk openly about costs and tradeoffs, or the business incentives of insurers and life science companies. Each was important, but none could be solved alone. A real solution would require something that could bridge all these issues and bring people together to reform our health care system in a way that no one group could do on their own. That solution didn't exist yet, and it wouldn't happen overnight, but I decided it was my time to try. It was time to leave academia, roll up my sleeves, and see how many partners I could find to build a way forward.

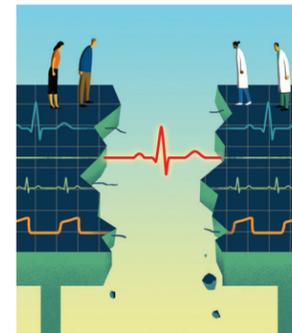
Thus was ICER born. A “laboratory” of a kind meant to experiment with new ways to cure a deep ethical wound at the heart of our health care system. A laboratory built to work with others also looking for ways to apply their beliefs and their hopes to build a health care system they could be proud of. A laboratory where good science, honesty, integrity, and mutual respect could combine to break down walls and create in their place a health care system that can guarantee all Americans fair prices, fair access to care, and future innovation.

That has been our vision for 15 years. And, due to extraordinary people, we have made remarkable progress from where we started. To these people – ICER staff, external policy and clinical experts, patient advocates, funders, leaders of visionary health care companies, devoted Twitter defenders, Board members – to these people ICER owes every step we have been able to take. It is with my most profound appreciation that I recognize their contributions. They are the heart of ICER.

Today, the better future for patients that we all yearn to see remains not the task of a single person or organization. It remains a project of many years, and one that will surely be marked by frequent disappointment. But the arc we are on is sure. Let us celebrate our progress and rededicate ourselves to our common work together towards a better future for patients – all patients.

Steven D. Pearson, MD, MSc
Founder and President
Institute for Clinical and Economic Review

The Status Quo is Broken.



The American health care system consistently fails to provide high-value care to all patients at a price they can afford. Neither the commercial market, nor government, has been able to address this inherent problem.

Our mission is to bring discussions about value, pricing, and insurance coverage into the open to help build stronger foundations for fair access to affordable care for all Americans. We perform reviews of the existing evidence, identify its strengths and limitations, and use cost-effectiveness analysis to suggest fair price ranges that align with the ability of health care interventions to improve the lives of patients and their families. We ask patients and patient groups to orient us in these efforts, seeking to capture what matters most to them about the risks and benefits of treatment, and to provide the context all policymakers should have about the lived experience of patients and their hopes for new treatments. We convene meetings at which patients can have their voice further amplified, and at which clinical experts, life science companies, and insurers can all learn from each other.

Our goal is to use the best scientific methods available to ground a more transparent process through which patients can be guaranteed fair prices, fair access, and a health care system primed for future innovation.

Fair Pricing. Fair Access. Future Innovation.



Since our beginning in 2006, ICER has worked on measuring how well new medical tests, treatments, and delivery system innovations work -- helping to identify what a fair price would be for that level of performance, and identifying how best to apply that evidence to insurance coverage so that patients receive appropriate access to the care they need. This reciprocal framework -- fair pricing coupled with fair access -- creates a grand bargain that disrupts the status quo, de-escalates the arms race between life science companies and insurers, and provides an opportunity for all participants in the health care system to benefit.

Today, ICER, in collaboration with policymakers, envisions a world where the healthcare system:

1. Delivers broad patient access to all therapies.

ICER wants to help policymakers use our findings to reward the most effective medicines with higher prices, while negotiating lower prices on the treatments that don't help patients as much.

2. Encourages investment into future innovation that can improve patients' lives.

While investors making bets on R&D need the potential of big financial returns, we believe that value-based drug pricing sends the right signal to the drug industry and venture capital firms about the kind of innovation we all want funded.

3. Minimizes wasteful spending of society's dollars.

If the US health system stops overpaying for new drugs that don't offer additional benefit, then it can afford to handsomely reward the new treatments that truly are transforming patients' lives.

Our Approach Makes a Difference.



Any discussion about health technology assessment must acknowledge the notion of tradeoffs. When society is forced to purchase a medical intervention at a price that far exceeds the intervention's ability to improve patients' health, there's a real opportunity cost involved.

And not just an economic cost, but a tangible health cost, too. When the entire US health system overpays for a treatment for some patients, there's a direct consequence: rising premiums, individuals dropping insurance coverage, and ultimately greater health losses for the patients who can no longer afford the care they need.

We want a health system that maintains sufficient incentives for the swing-for-the-fences innovation that can make a real difference for patients. But it's simply not sustainable to provide those same incentives for any new drug, regardless of efficacy. For the US health system to deliver high-value health care for all Americans, we need to find the right balance.

The Four Pillars of Actionable Value

ICER measures how well prescription drugs or health interventions work for patients and we suggest a fair price. Unlike any other organization, we actively solicit feedback from patients and families in addition to input from clinicians, manufacturers, and payers to inform our work.

1

Independence.

ICER's drug assessments are funded exclusively by non-profit foundations, government contracts, subscription revenue, and unaffiliated philanthropists. This legacy of independence – free from conflicts of interest from either the pharmaceutical or insurance industries – ensures that ICER's conclusions are objective, credible, and applicable for public policy.

2

Rigor.

Partnering with leading clinical experts and health economists from around the country, ICER reviews all relevant real-world and clinical data to gain a clear understanding of the net health benefit and lifetime cost implications of a new therapy compared to those that are already on the market. While commercial health plans may make coverage decisions after a cursory review of the evidence of several different therapies during a single meeting, a typical ICER assessment lasts eight months, incorporates public comment, and is ultimately published in a peer-reviewed journal.

3

Engagement.

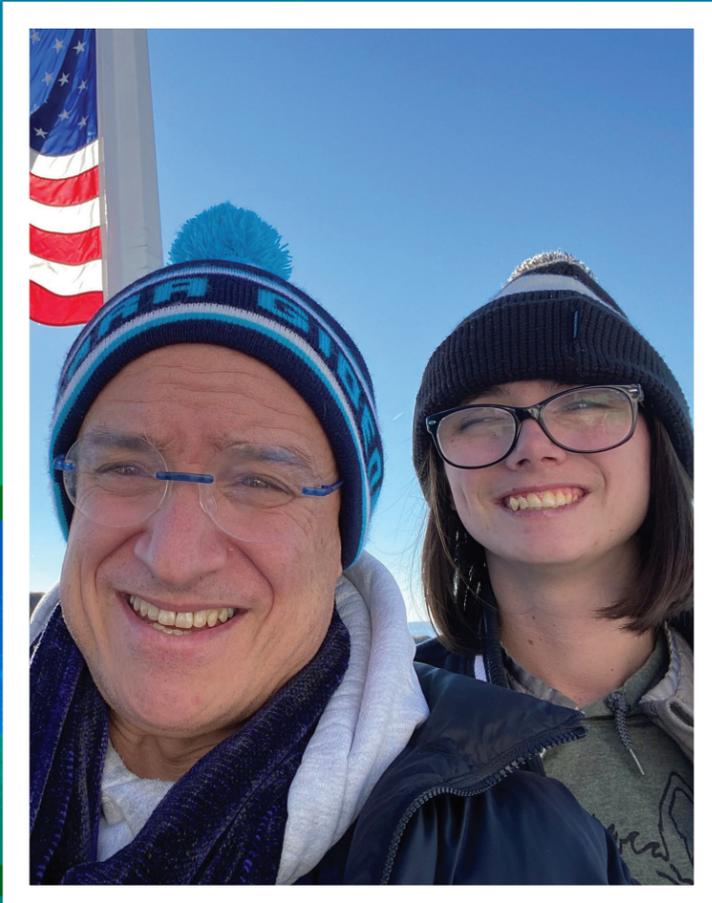
Throughout ICER's assessment process, we engage with clinical experts, patients and caregivers, and the relevant manufacturers to better understand the nature of the disease, the outcomes that matter most to patients, and the new treatment's anticipated role in a therapy pathway. There are multiple opportunities for both private feedback and public comment throughout our process, including at a public meeting where an independent appraisal committee votes on the therapy's benefits and long-term value, and where a panel of cross-stakeholder experts provide policy recommendations for coverage, future research, and advocacy.

4

Transparency.

ICER's eight-month value assessment process is iterative, open to public comment in multiple phases, and is highlighted by a public meeting where all of the evidence is presented, alongside testimony from patients, clinicians, manufacturers, and other stakeholders. All of our assessments, including any disagreement from external individuals and groups, is documented on our website in perpetuity, available to ground a more transparent process through which patients can be guaranteed fair prices, fair access, and a health care system primed for future innovation.





“As the father of a 19-year-old who lives with CF, I have rejoiced at the remarkable progress that has unfolded in treating this life-shortening genetic disease.”

The Patient Perspective

By Don Kreis
Patient Advocate, Cystic Fibrosis

Cystic Fibrosis (CF) is sometimes referred to as the “greatest story in medicine” and, as the father of a 19-year-old who lives with CF, I have rejoiced at the remarkable progress that has unfolded in treating this life-shortening genetic disease just in her lifetime. Indeed, I am convinced that the biggest threat to my daughter’s longevity is no longer CF itself.

Rather, my biggest fear is that civilization itself will fail her. The obvious fault line is the high cost of CF care, especially the recently approved breakthrough “modulator” medications that for the first time treat the cellular defect that causes CF.

Thus I was delighted to discover that ICER existed and was conducting rigorous examinations of the cost-effectiveness of these drugs.

In October of 2019 I was among the 5,000 people who celebrated at the North American CF Conference in Nashville when the FDA approved Trikafta, making a modulator treatment available to 90 percent

of people with CF. Tears flowed as we all sang along with Francis Collins, the guitar-playing director of the National Institutes of Health who played a key role in tracing the genetic roots of CF nearly three decades earlier.

Given that Trikafta rolled out with a list price in excess of \$300,000 a year, I am sorry there was no similar celebration when ICER released its detailed report on this drug along with the conclusion that the benefits of Trikafta did not justify its price. This truth was difficult for many in the CF community to hear.

That makes me all the more grateful to ICER for telling it, so comprehensively, persuasively, and compassionately. As it happens my daughter is in the 10 percent of Cystic Fibrosis patients whose miracle is still in the future. When it comes, we will need the cost to be sustainable for society to bear. I’m convinced that ICER will play a key role making it so.



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The Patient Perspective

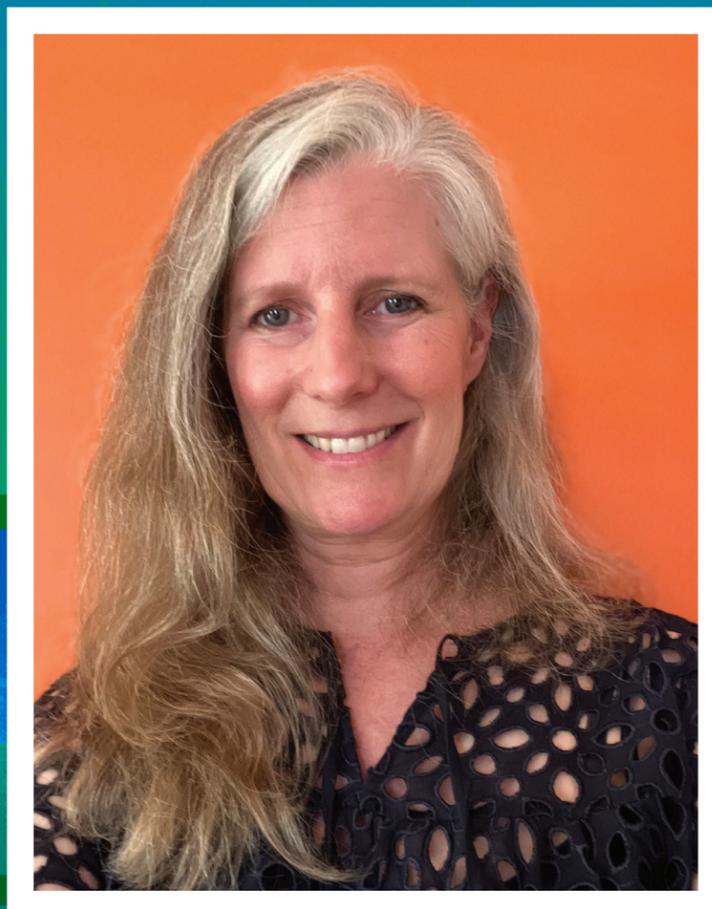
By Sneha Dave
Executive Director, Generation Patient

I have been a patient since the age of six. I also serve as the executive director of Generation Patient, a nonprofit focused on empowering adolescents and young adults with chronic conditions. Throughout the last couple of years of engaging with ICER, I have greatly appreciated the independence of thought and assessment — something that feels like a lost art in a time where there is pervasive industry influence over critical decisions affecting the patient community.

Given that patients in the U.S. grapple with the high prices of drugs, often unjustified, ICER has a growing role to continue its assessment of the price and rigor of the evidence. As patients, we need medical

innovation, but value assessment is a method by which we can seek accountability for truly novel innovation, at a fair price.

Further, as an independent voting member of the Midwest CEPAC, I look forward to the continued impact on our patient community to ensure that there is balance in all decisions. ICER's growing patient engagement over the last few years is exciting. I have no doubt that the next fifteen years and beyond at ICER will center on patients' experiences with an emphasis on health equity to ensure that whatever medical products are created can also be appropriately affordable for all patients.



“I have learned that there is an opportunity to bust through some of the tension between patient advocacy communities and what ICER traditionally has been.”

The Patient Perspective

By Rebecca Kirch

Executive Vice President, Health Care Quality and Value for the National Patient Advocate Foundation (NPAF)

While working on ICER's independent appraisal council over the last year, I have learned that there is an opportunity to bust through some of the tension between patient advocacy communities and what ICER has traditionally been. In my experience working on ICER's panel based in New England, I have seen an actual and genuine interest in involving patients and caregivers at the table.

The ICER deliberations are focused on the true north of what patients and families hold dear, and I've seen that opportunity play out in several ways. First, the invitation for patient advocates

to be on panels is significant, and shows that ICER values our input as much as that of physicians or health policy experts. Second, ICER features patient stories at the beginning of every meeting to ground the discussion. That context is crucial because it demonstrates how patients and caregivers contribute — and how important it is to juxtapose their lived experiences with the clinical and economic discussions. It's one of the best ways of marrying everybody's expertise for the shared goal of better outcomes.

Milestones to a More Equitable and Sustainable Health System

2006

- As a research program at Harvard Medical School's Department of Population Medicine, Steve Pearson merged the methods of epidemiology and ethics to create ICER, a laboratory for public dialogue on access and pricing in the US health care system.



2010

- ICER receives a major federal research grant to launch its first independent appraisal committee, the **New England Comparative Effectiveness Public Advisory Council (CEPAC)**.

2013

- ICER receives major funding from the Blue Shield of California Foundation and leaves academia to become an **independent, nonprofit research organization**.



- ICER assumes leadership of the **California Technology Assessment Forum (CTAF)**, which becomes ICER's second independent appraisal committee.

2014

- ICER assesses the new hepatitis C therapies **Sovaldi®**, and **Harvoni®**, attracting national attention for the rigor and public transparency of its HTA process, and leading to formal acknowledgment by major insurers that ICER's reports were informing their formulary decisions and pricing negotiations.



2015

- ICER assesses the new class of high cholesterol drugs known as **PCSK9s**, recommending its first formal "value-based price benchmark." ICER's price recommendation is more than 50% lower than initial list pricing.



- ICER formalizes the first public iteration of its Value Assessment Framework, establishing ICER as an international leader in methods development for health technology assessment.

- The **Laura and John Arnold Foundation** (now known as Arnold Ventures) provides a transformational financial grant expanding ICER's research impact.

- ICER launches the **Midwest CEPAC** as its third independent appraisal committee.

2017

- The **Department of Veterans Affairs** publicly announces its use of ICER's research to expand access to high-value therapies while achieving greater savings for taxpayers.



2018

- Two years following ICER's initial report, drug makers **Regeneron** and **Sanofi** reduce the price of their PCSK9 drug by more than 50% to reach ICER's benchmark range, leading to broadened access from many insurers.

- New York State** initiates a public process – anchored by public meetings where ICER analyses were presented – to negotiate supplemental Medicaid rebates on high-cost drugs without any terms that would limit patient access. Massachusetts has since adopted a similar process for Medicaid negotiations.

2019

- Novartis** prices its new gene therapy **Zolgensma®** at \$2.1 million, within the upper limits of ICER's value-based price benchmark, and far lower than the \$4-5 million range the manufacturer had previously discussed.

- The **Centers for Medicare and Medicaid Services (CMS)** cites ICER's favorable assessment of CAR-T therapies in its determination to expand coverage for Medicare beneficiaries.

- ICER publishes its first report on **Unsupported Price Increases** – Americans spent an extra \$4.8 billion on drugs having no new evidence to support their price increases.

2020

- The **National Academy for State Health Policy** drafts model legislation for states to implement legislation based on ICER's reports of Unsupported Price Increases.

- Early in the COVID-19 pandemic**, ICER published a white paper, hosted a series of webinars, and conducted a rapid assessment of the first emerging therapy for patients hospitalized with COVID-19, helping frame the national debate about what constitutes "fair" pricing for novel treatments in a pandemic.

- ICER launches **ICER Analytics**, a new cloud-based platform that will revolutionize the ability of payers, life science companies, patient groups, and others to put ICER reports into action.



2021

- ICER's white paper highlights several opportunities to strengthen the FDA's accelerated approval pathway, including ways to ensure rapid confirmatory trials and other measures woven into new FDA legislation.



- ICER's report on the controversial **Alzheimer's treatment Aduhelm®** sets the stage for a national debate. ICER demonstrates its unique role in being able to: 1) independently evaluate the clinical evidence when the FDA's internal disagreements raised doubts; 2) convene a transparent, public discussion with all stakeholders in the room; 3) set the benchmark for how the treatment could be fairly priced; and 4) establish guidelines for coverage and future research.

- ICER publishes its first annual **Assessment of Barriers to Fair Access** report, resulting in changes to insurance coverage policies that improve access to fairly priced drugs, and highlighting the need for greater transparency regarding how insurers frame and implement their coverage policies.



2022

2021

2020

2019

2018

2017

By the Numbers



93% of US payers agree that the US needs an independent Health Technology Assessment body



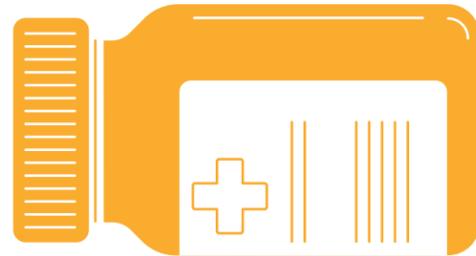
35 number of different state Medicaid departments that review comparative effectiveness studies from ICER to determine coverage criteria



5 The number of different ICER assessments focused on tackling the nation's opioid epidemic

\$2.1 million

The price of the most expensive therapy ICER has found to be cost-effective, demonstrating that value-based pricing will not erode incentives to develop transformative medicines



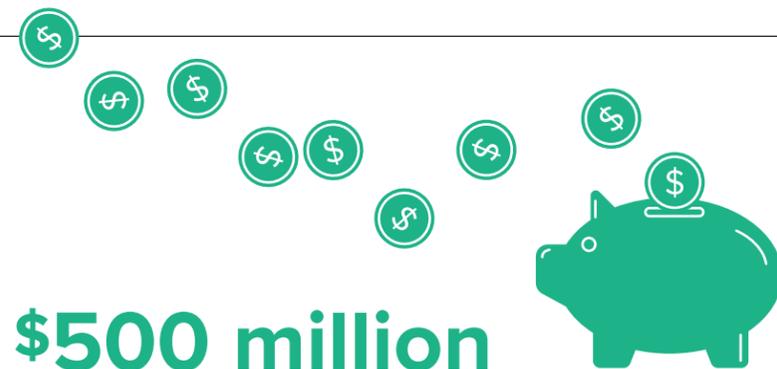
100% The percentage of surveyed payers (n=50) who agree that ICER will become "more influential" within the pharmaceutical industry over the next five years

300+

300+ The number of patients and patient group representatives with whom ICER has engaged

130+

130+ peer-reviewed publications have been authored by ICER employees



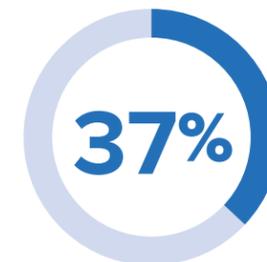
\$500 million

\$500 million The total amount saved by New York Medicaid by negotiating supplemental rebates based on a process that is anchored by a public meeting where ICER analyses are presented

16%



16% The median price discount needed to reach ICER's benchmark price when ICER's recommendation is known prior to a drug's launch



37% The median discount needed to reach ICER's benchmark price when the manufacturer launches without knowledge of ICER's recommended price



125

125 The number of publications that have been cited 3,579 times (this includes only citations of articles published in journals).

28%

28% The percentage of assessed therapies ICER has found to be cost-effective in the US

Expanding Fairness, Equity, and Transparency

By Sarah K. Emond, MPP

Executive Vice President and Chief Operating Officer
Institute for Clinical and Economic Review



The U.S. has a dysfunctional system, where a black box of medical innovation, corporate profit, monopoly prices, manufacturer rebates and insurance policies drive prices beyond what patients can afford and budgets can support. We are spending far too much for too little. As much as Americans need new and innovative ways to ease suffering and improve health, America needs new and innovative ways to ease prices and bring the cost of care in line with how

well it works. It's time to empty the black box and set prices based on evidence. Other countries do this. We can, too.

Many said the goals of ICER were too difficult to achieve. More than a few said ICER would never work, and some even actively organized against our efforts. However, today, we can acknowledge the outsized impact a 40-person, \$10 million organization made to meet the greater demands of a U.S. health system increasingly reliant on measures of evidence-based value. As Congress, state legislatures, private and public payers debate policy solutions to escalating costs, calling for transparent evidence to justify high prices, ICER's reports and recommendations are increasingly seen as the evidence-based gold standard on which to build a system of value-based health care.

We could not have accomplished any of this without our dedicated staff, patient participants, independent appraisal councils, advisory committees, funders, and our Governance Board. From the support from the National Pharmaceutical Council way back when we started, to the support of the Blue Shield of California Foundation that allowed us to become an independent non-profit, to the California Health Care Foundation and Arnold Ventures for their continued support, we thank you for the role you play in helping us push the United States toward a system of fair drug pricing and fair patient access.

There's still work to do, and we depend on your continued support. We are poised to continue our leadership in the fight for evidence and transparency in the American healthcare system. As we grow, we are hoping to substantially expand our community of researchers, economists, and stakeholder liaisons. We will expand the work we do analyzing the fairness of payer coverage policies. We will support our nation's small and large businesses to deliver better, more affordable health care to their employees. And we will continue to be a guide to our nation's state and federal leaders who are pushing for a more sane health care system, all in service to our goal to make sure patients get access to the care they need at a cost they and the health care system can afford.

To the next 15 years.