PIONEER HEALTH

The QALY and Cancer Treatments:
An Ill-Advised Match

By Dr. William S. Smith

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Table of Contents

Introduction

Cancer is a dreaded disease — perhaps the most dreaded. While the CDC reported that 659,041 Americans died of cardiovascular disease in 2019, cancer deaths were a close second at 599,601.[1] Despite being the second leading cause of death, cancer seems to strike with a certain randomness, many times impacting seemingly healthy people whose lifestyle, in many cases, appears not to be a factor in the onset of the disease.

Cardiovascular disease treatments and oncology treatments increasingly differ in their costs to the healthcare system. One recent study of healthcare claims data listed oncology as the number one therapeutic area for high-cost claims, making up 18 percent of such claims. While there are some high-cost cardiovascular treatments, such as newer anticoagulants, many commonly prescribed cardiovascular treatments, such as numerous statins and antihypertensives, are low-cost generics. This same study pointed out that oncologics were the most costly therapeutic class (non-discounted spending), with spending rising from $39.1 billion in 2015 to $67.5 billion in 2019.[2]

While high spending levels on cancer treatments is certainly an issue for the healthcare system, denying afflicted patients' access to innovative cancer therapies can be a political third rail for healthcare policymakers. Most people know someone who has suffered with cancer and they know the emotional fear and the physical pain that can follow such a diagnosis. Denying treatments to these patients can be viewed, literally, as enforcing a death sentence.

Nonetheless, policymakers have waded into these perilous waters by supporting "health technology" assessments, or studies, that seek to weed out oncology therapies that are deemed "not cost effective." In the United States, the Institute for Clinical and Economic Review (ICER) has "reviewed" a number of new cancer therapies and, in most cases, argued that these medicines were "not cost effective." According to one study, ICER concluded that seven of the newest medicines should not be placed on health plan formularies unless they offered discounts of between 51 and 68 percent.[3]

President Biden has said that he learned, when he was head of the Obama administration's "Cancer Moonshot," that many cancer therapies are "abusively priced by manufacturers." Therefore, he has promised to have an "independent review board" assess the value of "specialized biotech drugs that have little or no competition to keep prices in check."[4]

Of course, by definition, this review board cannot possibly be "independent" as the President is publicly arguing that the board's mission will be to cut the costs of biologics, especially cancer therapies, for payers such as the government. The political danger for the President will be the danger that accrued to British policymakers when, in 1999, they created the National Institute for Health and Care Excellence (NICE) to hold down drug costs in Britain's National Health Service (NHS). By 2010, a political firestorm had developed when it became clear that NICE's devaluation of new cancer therapies, and the denial of those treatments to the British people, had transformed the quality of Britain's oncology care into the worst in Europe.

This lengthy narrative of Britain's experience with NICE is an important prologue to understanding the political dynamic that can occur when governments embrace cost-effectiveness reviews for oncology products.

The UK's Cancer Drugs Fund

The Quality Adjusted Life Year (QALY) criteria that are so controversial in rating the costs of cancer therapies was a British creation. Alan Williams, a professor of health economics at the University of York, began developing the QALY in the late 1960s and 1970s.[5] The QALY rates the value of a therapy according to its ability to extend life and to improve the quality of life. Many breakthrough cancer therapies may only extend the life of the patient by months, not years. For this reason, new cancer therapies have tended to get poor ratings in systems that use the QALY, such as NICE and ICER.

By the mid-1990s, many British health policy experts, including members of the House of Commons' Health Committee, had embraced the idea that there are "four hurdles" a pharmaceutical needed to cross before it should be made available to the British public. To the traditional three hurdles — safety, quality of manufacture, and efficacy — a fourth hurdle was added, cost effectiveness. Yet, at this early stage, how cost effectiveness was to be measured was not determined, and pharmaceutical companies began hiring health economists to make the case that their drugs were cost effective.

As money got tighter in the National Health Service (NHS), health policymakers began to talk openly about the need to "ration" treatments. In fact, over the course of the 1990s, the famous and influential *British Medical Journal* published 45 articles that discussed rationing. Had not "rationing," after all, been a huge success in allocating resources during the Second World War? Throughout John Major's tenure as Prime Minister, the debate over funding for, and the sustainability of, the NHS was intense.

In a prelude to the creation of NICE, in 1995, then-Minister of State for Health Gerry Malone was asked to decide if the NHS would pay for Beta-interferon, a treatment that seemed to lessen the severity and frequency of attacks suffered by multiple sclerosis (MS) patients. The cost of Beta-interferon for all of Britain's 70,000 MS patients was estimated to be as much as £380 million, or 10 percent of the total NHS budget.

Malone compromised on the decision, allowing the use of

Beta-interferon but discouraging its widespread use. Malone, however, was very unhappy about being forced to make such a life-and-death decision, and he tasked his staff with developing a system whereby politicians such as himself would not be placed in this difficult position again. While NICE was not created by Malone's team, it was during his tenure that the idea of some type of cost effectiveness referee gained traction among British policy makers.

Then came Tony Blair's landslide election in 1997 on a platform to "save the National Health Service." One way to accomplish this was to reduce the NHS's purchase of "ineffective" treatments, and create of some type of body to rate the value of treatments. The trade association for pharmaceutical firms, the British Pharmaceutical Industry (BPI), generally supported such a body because the NHS had a decentralized system of pharmaceutical access, with some regions permitting the use of new drugs and others restricting them. The BPI felt that a competent body of professionals would make access to new drugs more uniform across the whole system. This was a profound misjudgment on the part of industry.

It was Frank Dobson, Blair's minister of state for health, who came up with the name "NICE," the acronym standing for the "National Institute for Clinical Excellence." In July of 1998, the government published *A First Class Service*, the road map for the use of NICE to "improve" quality in the NHS by bringing "authoritative guidance… for all health professionals on the latest drugs and technologies."[6]

The reason that ICER does not include the clear societal benefits of certain treatments is obvious: consideration of such data would result in more treatments being rated as cost effective.

Initially, British policymakers were not sure if NICE would increase or decrease NHS spending. The hope was that it would rationalize spending and steer the healthcare community toward the most cost-effective treatments, thereby increasing quality and affordability.

So, NICE began doing reviews and rendering judgment on therapies such as the removal of wisdom teeth, flu treatments and coronary stents. NICE first got into hot water in 2000 when it judged two treatments for multiple sclerosis, Beta-interferon and glatiramer acetate, as not worth the "benefit and costs." The result was exactly the kind of firestorm Gerry Malone had sought to avoid in his earlier decision on Beta-interferon. MS patients in wheelchairs swarmed the House of Commons, and the MS Society took out newspaper ads condemning the decision. NICE held fast, but Tony Blair was not happy with the political fallout, so he instructed NHS to negotiate with manufacturers and, with a price cut, some access was restored to patients.

NICE began to get an international reputation with payers around the world who were accessing their website and reading their assessments. Then, came Herceptin. The novel breast cancer drug had its clinical trials cancelled early because the data were so strong; it was viewed as unethical to keep people in trials taking placebo. The head of the American Society of Clinical Oncology (ASCO) declared Herceptin to be the greatest breakthrough in breast cancer treatment in 30 years.

While Herceptin had been approved for end stage breast cancer and metastatic breast cancer, it had not yet been evaluated by NICE for early stage breast cancer. And British women wanted it, despite the fact that its manufacturer had not even applied to the EU for a license for early stage cancer. Nonetheless, the stories of these women, desperate for Herceptin, filled the media. Petitions were delivered to 10 Downing Street and marches on Parliament were organized.

The media narrative on Herceptin became twofold. First, the NHS had overspent its budget so it could not pay for the breakthrough drug. The second, and more plausible problem, was that NICE was terribly slow at conducting evaluations, sometimes taking more than two years to review a new drug. Reviewing cancer therapies, in particular, tended to be slower since NICE wanted to collect data on overall survival (OS) rates, data that did not typically emerge from clinical trials because of the extended time period generally required to determine length of survival.

With this political pressure, the politicians gave in. The Health Secretary ordered the NHS to give Herceptin to early stage patients in order to evaluate its effectiveness. However, the leaders of NICE as well as other health policy experts, viewed this decision as an emasculation of NICE by politicians who were agreeing to offer a drug without a review or a license due to political pressure. The tabloid press disagreed, and accused NICE of literally killing patients.

As the battle raged, the EU finally provided a license for early stage breast cancer and the NHS agreed to cover it. The Herceptin controversy, however, was a window into what was to come.

The next struggle was over an Alzheimer's drug, and then a treatment for macular degeneration. With each review, it became clear that the QALY had become the standard instrument of assessing value and that the "threshold" for rating a drug as cost effective, £20,000–£30,000 per QALY, was likely to result in determining many new drugs as not cost effective.

With the NHS budget growing, and pharmaceutical companies developing ever-more expensive cancer therapies, there was pressure on NICE to act. NHS began delaying payment for new oncology products until NICE had completed its reviews. Furthermore, the NHS — unwisely it seems — informed patients that if they paid for any of these yet-to-be approved drugs out of their own pockets, the NHS would not pay for any of their overall care. When the media discovered

incidents, the stories put the NHS in a very unfavorable light.

NICE also conducted a review of four new kidney cancer drugs and although the drugs were ruled as "clinically effective," they were ruled as "not cost effective" because they violated the QALY thresholds. After these kidney cancer reviews, the political dam was broken by a study of 14 countries' spending on cancer care compared with Great Britain. The report concluded that, for Great Britain to spend as much as the average per capita on cancer care, the NHS would need to increase its spending on cancer care by £200 million per year.

Conservative Party politicians quickly proposed a "Cancer Drugs Fund" that would pay for new cancer drugs whether or not NICE had reviewed them. The Cancer Drugs Fund was allocated a budget, probably not coincidentally, of £200 million. The Conservative Party leader, David Cameron, announced the idea for the Cancer Drugs Fund in 2010 after learning the plight of a constituent who had been denied a kidney cancer drug.

Embraced by physicians, patients, and the pharmaceutical industry, the Cancer Drugs Fund was a great political success. While many economists and NICE supporters were downright hostile to it, arguing that it was an inefficient waste of money, the reality is that it won the support of the British public because NICE, and its use of the low-threshold QALY, had degraded the quality of cancer care in Britain. David Cameron could say, with some truth, that "other European countries are doing better than us at giving people longer, happier lives with cancer…and in the UK today there are some people — some thousands of people — who want a certain drug, whose doctors tell them they should have a certain cancer drug, who don't get it."[7]

This lengthy narrative of Britain's experience with NICE is an important prologue to understanding the political dynamic that can occur when governments embrace cost-effectiveness reviews for oncology products. The first dynamic was demonstrated by Gerry Malone: politicians do not want to make decisions to deny specific therapies to patients. Instead, they want to fob those decisions onto an "independent" body of experts who can assure the public that the decision was made based upon "objective" criteria.

The second dynamic, most prominent in government-funded healthcare systems, is that the entities charged with assessing cost-effectiveness increasingly lean toward denying access to new medicines. Government healthcare programs tend to exhibit budgetary stresses because free or low-cost care tends to drive higher utilization. Therefore, governments create "cost effectiveness" functions to justify limiting utilization of new technologies. The entities charged with assessing cost-effectiveness understand that their mission is to label certain purchases as not cost-effective and that their role is not to justify increases in spending. In short, the deck becomes stacked toward restricting access to new medicines.

These are useful lessons in understanding ICER's assessment of the value of oncology treatments, as ICER methodology tends to rate cancer drugs in a way that discounts their value. The Cancer Drugs Fund story is a history that probably should be reviewed by policymakers in the Biden administration who seem to be embracing a cost-effectiveness review of biologics — likely to include many new cancer drugs.

Problems with ICER and the QALY in Evaluating Oncology Therapeutics

The use of the QALY has multiple infirmities that become apparent when reviews of oncology products are published. While the withholding of cancer treatments based upon economic modeling has serious ethical implications that are obvious, this paper will largely discuss the methodological and contextual shortcomings of the QALY in evaluating cancer therapies.

1. The Societal Benefits of Effective Oncologics: A Disease of Families

When a person is a diagnosed with cancer, the burden is not simply on the patient, but falls on the whole family. Studies indicate, for example, that a typical caregiver for a cancer patient provides an average of, a staggering 32.9 hours of care per week.[8] Not unexpectedly, this takes a huge toll on these caregivers. They experience very high rates of depression, anxiety and insomnia. [9, 10, 11]

One would expect the value of any therapy that ameliorates the tremendous burden on caregivers to reflect this significant benefit. Yet, ICER does include "indirect" benefits in their value assessments. Instead, over the years, as it became apparent that assessing no value to caregiver sacrifices seemed flawed and ill-conceived, ICER conceded the value of some of these social benefits yet did not actually incorporate that value into its models. ICER obliquely and opaquely hinted it may consider the societal benefits of a therapy when the societal cost is large.[12] In other words, patient advocates should try to make the case that these caregivers matter, and ICER may consider it. But, no promises. ICER does this in many situations when its frameworks have obvious flaws, such as low QALY thresholds for rare disease drugs, where it has hinted at considering higher thresholds for certain therapies. The more inferred concessions are made by ICER, the more subjective its frameworks appear.

> This study argues that ICER's methodology therefore discriminates against people of color in assessing the value of therapies that this patient population desperately needs.

The reason that ICER does not include the clear societal

benefits of certain treatments is obvious: consideration of such data would result in more treatments being rated as cost effective. For example, one study demonstrated that the inclusion of economic productivity gains resulting from a given therapy would result in a higher value for the therapy. "The exclusion of productivity costs can alter, often underestimating, the assessment of value."[13] That same study pointed out that although ICER has nodded to the inclusion of societal benefit values in its reviews: "Since March 2017, 18 of 19 (94.7%) pharmaceutical value assessment reports from the Institute for Clinical and Economic Review included productivity costs in the report, but only 2 (11.1%) included productivity in a primary (co-base case) analysis."

Patient groups, patients and their families who get frustrated at the dodginess of the ICER framework need to keep in mind that ICER is not an independent, objective actor. ICER was originally formed by the health insurance industry with one goal in mind: lower its drug costs to increase its profits. ICER's roots therefore are not found not an academic body seeking to ascertain the true "value" of therapies; all its cost effectiveness reviews need to be understood in this light.

There seems to be however another reason why cost-effectiveness studies are not based upon patient experiences whenvaluing quality of life improvements. It is because patients would likely value these improvements more highly than the general public, leading, in the final analysis, to more therapies being rated cost effective.

2. Oncology Clinical Trial Results Do Not Reflect the Patient Population Who Will Use the Medicine and Measure Progression-Free Survival (PFS), not Overall Survival (OS)

ICER cost effectiveness reviews are typically based upon clinical trial data. This is a particular problem when evaluating cancer treatments since the clinical trial population in an oncology trial is not representative of the population who will actually take the medicine. This is the case because, according to a very recent report from the Congressional Research Service, "research has shown that more than half of oncology drug use is off-label."[14] When drugs are used off-label, by definition, they are not being used for the conditions for which they have been FDA-approved and labeled. Consequently, the data that ICER uses to conduct a cost-effectiveness review on a treatment is projectable to less than half of the population that will actually use the medicine.

Not only is ICER studying data from patients who do not represent the patients who will use the medicine, the clinical trial exclusion criteria allows for the accrual of a very select group of patients. The co-morbidities that preclude trial eligibility are often those that afflict many cancer patients who are typically elderly and at higher risk than the study population. As Richard Pazdur and his coauthors commented: "Although eligibility criteria are needed to define the study population and improve safety, overly restrictive eligibility criteria limit participation in clinical trials, cause the study population to be unrepresentative of the general population of patients with cancer, and limit patient access to new treatments."[15]

Moreover, the QALY methodology used by ICER measures Overall Survival (OS), or how long a patient survives, while most oncology clinical trials measure Progression Free Survival (PFS). Therefore, economic evaluations of oncology must make guesses about OS based upon PFS data in the trial. As one study put the problem, "QALYs relies on evidence of improvements in overall survival (OS), and clinical trials rarely run long enough to establish that… Consequently, the use of clinical trial evidence in cost effectiveness studies requires analysts to estimate OS on the basis of intermediate endpoints such as PFS, time to progression, etc."[16] In other words, the QALY is ill-suited to value cancer therapies with clinical trial data alone.

One new study[17] has pointed out that oncology clinical trials display considerable underrepresentation of people of color, calling into question QALY studies that rely on clinical trial data. "Oncology is particularly illustrative of this systemic inequity. In a review of recruitment in phase 3 cancer clinical trials conducted between 2001 and 2010, reviewers found that just 6.2% were African American; in contrast to studies conducted between 1990 and 2000, in which 10.5% were African American. This illustrates that despite awareness of the problem that RCTs are highly homogeneous, efforts to enroll African Americans appear to have diminished or become less effective over the last two decades."

This study argues that ICER's methodology therefore discriminates against people of color in assessing the value of therapies that this patient population desperately needs. "This failure to power clinical trials to yield statistically significant results for patients of color compromises the clinical validity of data and information regarding disease presentation and therapeutic responses and findings regarding safety and efficacy. By extension, methodologies designed and employed by health economists to assess the relative value of health technologies are equally compromised and discreditable."

3. Discounting of Patient Preferences

When estimating QALYs, as has been discussed, cost-effectiveness studies assess the ability of a treatment to extend life and to improve the quality of life. Measuring health-related quality of life (HRTQoL) is a two-step process. First, patients are given"patient reported outcomes" (PRO) questionnaires, in which they report changes in their quality of life. From the perspective of a cancer patient, the next step is where the process is fundamentally flawed. "Weight…is attached to

each aspect of HRQoL ***from the perspective (by convention) of the general public***, in order to summarise the PRO data provided by patients."[18]

In other words, the value assigned to any improvements in various aspects of a patient's quality of life are not provided by surveying patients themselves who live with their illness, but by members of the general public, who must imagine the value, for example, of a substantial reduction in pain. The rationale for using general population opinions is that, "the views of the general public, as taxpayers and potential patients…should be taken into account in decisions which affect the allocation of public sector budgets…"[19]

There seems to be however another reason why cost-effectiveness studies are not based upon patient experiences when valuing quality of life improvements. It is because patients would likely value these improvements more highly than the general public, leading, in the final analysis, to more therapies being rated as cost effective. Surveys of the general public do not accurately reflect the values actual cancer patients tend to place on incremental improvements in their quality of life, because patients tend to value improvements in their health more highly than do non-patients.

As explained by the Office of Health Economics, London, UK, "the practice of using valuations of members of the general population, as recommended by NICE, is problematic because such individuals typically display a misunderstanding of what it is really like for patients to live with cancer."[20]

One large study of the differences between evaluations made by the general public and patients concluded that surveys of the general public tended to accord less value to treatments that may extend life than to therapies that restore performance: "the use of general population values rather than patient values tend to favour interventions aimed at achieving perfect functioning whilst disfavouring life-prolonging interventions."[21] For obvious reasons, discounting life-prolonging interventions is contrary to the goals of most patients with cancer.

4. The Challenge of Personalized Medicine

With the mapping of the human genome, medical science is discovering that many disease states are gene-related. This is particularly true in oncology, where patients with certain genetic profiles respond differently to different therapies. For some patients, a particular therapy is highly effective and has few side effects, while for other patients, it does not work at all. As noted on Cancer.net, the patient information website of the American Society of Clinical Oncologists (ASCO): "researchers began finding genetic differences in people and their cancers. These differences explained a great deal about why cancers responded differently to the same treatment."[22]

Genetically targeted cancer therapies, often referred to a "personalized medicine" are becoming more and more common. The ASCO website mentions that targeted treatments are available for the following cancer types: bladder cancer, brain cancer, breast cancer, cervical cancer, colorectal cancer, endometrial cancer, esophageal cancer, head and neck cancer, gastrointestinal stromal tumor (GIST), kidney cancer, leukemia, liver cancer, lymphoma, lung cancer, melanoma, multiple myeloma, neuroblastoma, neuroendocrine tumors, pancreatic cancer, prostate cancer, soft tissue sarcoma, stomach cancer, and thyroid cancer.

The Personalized Medicine Coalition calculated that 25 percent of drug approvals between 2015 and 2018 were for "personalized medicines."[23] It pointed out to ICER that "A population-level framework may encourage the restriction of access to a new drug based on reported averages, which limits treatment options available to individual patients who may have benefitted from them."[24] In other words, if personalized medicines are deemed not cost effective by a value framework, access may be prevented even for patients who, because of their genetic profile, may respond exceptionally well to a certain therapy. As a study in the *Journal of Clinical Pathways* put the problem: "If the application of the framework is not sufficiently personalized, then the value determination applies only to the average patient, not specific ones."[25]

> Given that progress against cancer is measured in months, not years, the QALY tends to be an inappropriate metric for new cancer therapies because their impact, while clinically meaningful, does not often extend longevity for years.

ICER has conducted multiple reviews[26] over a number of years on treatments for multiple myeloma. After its first review in 2016, the Multiple Myeloma Research Foundation pointed out "the promise of precision medicine is that each patient is unique and will consequently respond to treatment differently based on their particular genetic profile and further understanding of the biology of their disease."[27]

In that 2016 review, ICER had a difficult time conducting its cost effectiveness reviews because of the varying profiles of multiple myeloma patients. After reading ICER's report, one physician concluded that ICER had "come to understand that each patient is unique and different and that all therapies will be required during the course of multiple relapses."[28] Despite this recognition however and the promise of the new breakthrough targeted-therapies that had become available by 2016, ICER concluded that "at current wholesale acquisition costs, the estimated long-term cost-effectiveness of these regimens exceeds commonly cited thresholds." According to ICER, none of the new breakthrough treatments were rated "high value."[29]

In 2016, ICER also released a report on treatments for non-small cell lung cancer which generated great criticism

from the oncology community. According to one group of highly-regarded oncologists: "Based upon our reviews of this report, ICER appears to represent a perspective that is less oriented towards patient benefit than towards motivations that would limit patient access to new therapeutic options. ICER's clinico-economic methods include approaches and metrics that, due to their singular focus on population-level health, would likely fail patients on an individual, clinical needs basis."[30]

Since these controversial 2016 reviews, ICER seems to put more focus on treatments for certain subgroups of cancer patients. However, as discussed below, this has not resulted in more favorable value assessments for targeted treatments. Between 2016 and 2019, ICER reviewed 11 new cancer therapies and concluded that only four were "cost effective."

5. QALYs Represent a Poor Measure of Progress Against Cancer

Progress against cancer is measured in increments. A key outcome metric is "progression-free survival (PFS)", which is: "The length of time during and after the treatment of a disease, such as cancer, that a patient lives with the disease but it does not get worse. In a clinical trial, measuring progression-free survival is one way to see how well a new treatment works."[31]

Typically, advances in PFS are measured in months, not years, so PFS9 indicates that for 9 months of a patient's life the cancer did not get worse.

Given that progress against cancer is measured in months, not years, the QALY tends to be an inappropriate metric for new cancer therapies because their impact, while clinically meaningful, does not often extend longevity for years. As noted by the authors of *Limitations of QALY: A Literature Review*, "The QALY metric has also been critiqued for having insufficient sensitivity to measure small but meaningful changes in health status — or utility. Such changes in health status are particularly applicable (and important) to certain patient subgroups, for example, cancer patients where multiple studies have outlined a need for additional dimensions to be considered."[32]

This tendency of ICER to undervalue cancer therapies was documented in a 2020 study published in *The Lancet* that examined ICER's cost effectiveness reviews completed between 2016 and 2019, for 11 cancer drugs. Of the 11 drugs, only four were rated as cost effective and therefore "recommended for formulary inclusion at the stated list price."[33] The study concluded that, for the other seven drugs to be recommended for formulary inclusion, manufacturer list prices would need to fall 51 percent to achieve the goal of $150,000 per QALY and 68 percent to achieve $100,000 per QALY.

Moreover, the authors of this *Lancet* study concluded that, because the UK's NHS can negotiate substantial discounts from manufacturers, its cost-effectiveness body, NICE, is more likely to rate new cancer drugs as cost effective than ICER. "Our analysis shows that NICE's capacity to negotiate price discounts and access schemes results in much lower cost per QALY valuations and more favourable recommendations than those of ICER for similarly assessed cancer drugs."[34] The study authors concluded that "the QALY, because of the way it is constructed, may indeed fail to accurately capture the value of the health gains that are deemed important by cancer patients."

Conclusion

Cancer death rates have been declining for a number of decades. Between 2001 and 2017, cancer death rates declined by 1.8 percent for men and 1.4 percent for women.[35] However, the COVID crisis could see that progress reversed. The American Society of Clinical Oncology reports that "Among Americans scheduled for a cancer screening test such as a mammogram, colonoscopy, skin check, and Pap/HPV test during the pandemic "nearly two-thirds, or 64%, reported that it was delayed or cancelled."[36]

In other words, we could see cancer death rates rise for the first time in decades. The only way to offset this increase is for Americans to return to regular cancer screenings, and to allow those diagnosed to have access to the latest, most advanced treatments. For those covered by payers who apply the ICER framework for evaluating cancer treatments, access would be precluded.

There seems to be a certain naiveté on the part of some policymakers, and some in industry; they who think ICER can be "persuaded" to accept major revisions in its framework.

The ICER framework could certainly be improved. Possible reforms include allowing actual patients to make value determinations, rather than members of the general public; including societal benefits in their primary analysis; and, raising QALY thresholds for certain serious cancer treatments.

The problem, of course, is that ICER is highly unlikely to accept such reforms. Its mission is not to conduct "objective" evaluations of treatments that could offer help and hope to very sick people. Rather, it seeks to limit patient access to expensive new treatments and reduce drug costs for payers.

There seems to be a certain naiveté on the part of some policymakers, and some in industry; they who think ICER can be "persuaded" to accept major revisions in its framework. Sadly, however, ICER's goal is not to adopt the best possible framework for patients and their families. Instead, ICER is focused creating leverage for payers in their pricing negotiations with manufacturers. Only certain very limited concessions are likely to be accepted by ICER in adapting their framework.

Given its unwillingness to accept reforms that are advocated

by patients, as well as the inadequacy of the QALY in evaluating the cost effectiveness of cancer therapies, policymakers should not waste time trying to convince ICER to modify their framework methodology. Instead, policymakers would be wise to adopt the David Cameron strategy of banning use of the QALY altogether in evaluating cancer treatments and prohibiting ICER from advising U.S. government agencies regarding their formulary design.

Some policymakers will recoil from this approach and argue that the cancer treatment cost growth is "unsustainable" and rigorous cost-effectiveness evaluations should be a priority. However, the data do not support unsustainable cost growth over the next five years. According to consulting firm IQVIA, "Brand losses of exclusivity are projected to have a $139 billion negative impact on brand sales from 2020–2024, compared to the $107 billion impact seen from 2014–2019." As a result, patent expirations over the next five years will make new oncologics quite affordable to the healthcare system. The IQVIA report also points out that: "Manufacturer net prices are expected to grow between 1% and -2% in the United States over the next five years, significantly below historic levels."[37]

The control and cure of cancer, to save lives in this and the next generation, can only be achieved through the discovery of novel treatments.

Given the possible growth in cancer incidence because of reduced screenings during the COVID pandemic, as well as the coming patent expirations on huge selling drugs such as Januvia, Humira, Victoza, and Vyvanse, the next five years is precisely the time to avoid restricting access and to incentivize companies to invest more in discovering better treatments. The control and cure of cancer, to save lives in this and the next generation, can only be achieved through the discovery of novel treatments. And, let's face it, restricting access to new therapies that can reduce suffering is both cruel and morally unacceptable.

Endnotes

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About the Author

Dr. S. William Smith is Visiting Fellow in Life Sciences at Pioneer Institute. He writes about public policy issues impacting the life sciences industry with particular emphasis upon pharmaceuticals. Dr. Smith has 25 years of experience in government and in corporate roles. He spent ten years at Pfizer as Vice President of Public Affairs and Policy. He later served as a consultant to major pharmaceutical, biotechnology and medical device companies, and was President of a small medical device company for three years. His career has also included senior staff positions for the Republican House leadership on Capitol Hill, the White House, and in the Governor's office in Massachusetts. He is affiliated as Research Fellow and Managing Director with the Center for the Study of Statesmanship at The Catholic University of America (CUA). He earned his PhD at CUA and a bachelor's degree from Georgetown University

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185 Devonshire Street, Suite 1101 Boston MA 02110 617.723.2277
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