Perspective on the Price of Innovative Prescription Medicines

Over the past four decades, the return on investment to society from the development of innovative medicines has been substantial. Biomedical discovery has resulted in better ways to prevent, detect, diagnose and treat such life-threatening or disabling diseases as cancer, diabetes, HIV, inflammatory conditions, multiple sclerosis, pulmonary hypertension and viral hepatitis. As a result, death rates are declining across disease groups, a number of previously fatal diagnoses have been transformed into manageable, chronic conditions and the discovery that genetic mutations are the cause of most cancers and many other diseases has led to increasingly precise therapeutics that are changing standards of care.

Yet, these revolutionary advances also come at a time when payers and some policymakers are questioning the price of innovative therapies and whether “specialty medicines,” which represent less than 4 percent of total healthcare expenditures, are putting a strain on the health care system. As the debate continues, health plans are increasingly denying coverage for novel therapies or requiring patients to pay a larger percentage of the medicine’s cost. Of added concern, some politicians are calling for legislation to regulate drug pricing without any understanding that for decades prescription medicines have remained one of the most sustainable sectors of total health care expenditures or how the value-created outcomes of innovative therapies impact patients, the health care system and the economy.

Because these potential actions can affect the health outcomes of millions of Americans, it is time for a reasoned discussion of the price and value of innovative therapies. Towards this end, the following information addresses the price of medical innovation, provides a comprehensive look at the relationship between new medicines and total health care spending, and documents the overall value of medical innovation to patients, the health care system and the U.S. economy.
When it comes to assessing the “value” of innovative medicines, it is important to start with a working definition of this term. In economics, “value” is defined as “the measure of the benefit of a good or service,” not the actual price. Therefore, health economists define the value of medicines as the health outcomes achieved—such as extra years of life, higher quality of life and fewer costly hospitalizations, invasive medical procedures and doctor’s visits per dollar spent.

Applying this definition, innovative medicines provide increasing measurable value to patients, society and the U.S. health care system, even as overall drug spending on a relative basis has remained constant for over 50 years. Using cancer as the case in point, new treatments have contributed to a 25 percent drop in cancer deaths over the last two decades, making it possible for patients to experience an aggregate of 23 million additional years of life and generate $1.9 trillion in economic activity.

Using colon cancer as an example of these tangible measures, a 2009 study by Cornell University researchers concluded that when longevity, quality of life and the greater efficacy of treatments are considered, the true cost of colon cancer medicines is 30 percent less than a decade earlier.

Reinforcing these conclusions, the Commerce Department’s Bureau of Economic Analysis (BEA) applied 2015 data to report that “the net value of treatment has grown substantially, consistent with medical technology, leading to better health outcomes at a lower cost per patient.” Between 2000 and 2010, the report found an increase in positive health outcomes from treatment with new therapies in 20 of the 30 chronic diseases studied.

Moreover, other studies measuring the value of new therapies reveal that:

- Treating newly diagnosed multiple myeloma patients with novel therapies increases the time before the disease progresses, producing significant cost savings. According to a recent study, “Total direct monthly costs per patient declined steadily over time, decreasing by 68 percent from the initial treatment to the period post 18 months.”
- According to the Alzheimer’s Association, the development of a new treatment that delays the onset of Alzheimer’s by 2025 could reduce Medicare and Medicaid spending on patients by $218 billion annually by 2050.
- On average, improved access to medicines through Medicare Part D program saves about $1,200 per year in hospital, nursing home and other costs.

The Congressional Budget Office (CBO) changed its scoring methodology in 2012 to reflect savings in medical spending from policies that increase the use of medicines by Medicare patients. Under this new methodology, CBO scores every 1 percent increase in the number of prescriptions filled with a .20 percent decrease in spending on other medical services, such as emergency room visits and hospitalizations.
Factors Affecting the Price of a Medicine

Because developing a new drug on average takes at least 10 years from the research phase through U.S. Food and Drug Administration (FDA) approval, novel medicines are expensive to bring to patients. According to the Tufts Center for the Study of Drug Development, the average cost today to develop one approved therapy is $1.4 –$2.6 billion for R&D costs and $1.2 billion in cost of capital during the 10-plus years a drug candidate spends in development. The Tufts study further finds that $312 million is spent on post-approval studies to test new indications, formulations and dosage strengths, bringing the life-cycle cost up to $2.9 billion.¹⁰

Factored into this price tag is the high cost of failure in drug development. Currently, less than 12 percent of the candidate medicines that make it into phase I clinical trials will be approved by the FDA.¹¹ Beyond these R&D costs, a range of elements go into the calculations behind drug pricing, including the value offered by a therapy with life-enhancing benefits. In the case of the hepatitis C virus (HCV), for example, the price of a 12-week course of an oral drug that results in a 95 percent chance of a cure for patients with certain genotypes is slightly higher than the 48-week regimen of once-a-week interferon injections that many patients have difficulty tolerating and does not cure HCV.¹²

Other factors affecting drug pricing include the number of years to gain approval, the size of the patient population, the severity of the condition, the time patients are on the drug, ongoing regulatory costs post-approval, the number of years of market exclusivity, the number of years to achieve reimbursement, intellectual property litigation and counterfeit expenses.
Prescription Medicines and Total Health Care Spending

Although much attention has been paid to the cost of medicines, retail prescription drugs account for less than 10 percent of total health care spending and innovative cancer medicines cost less than 1 percent\(^\text{13}\) of total health care spending. Moreover, the percentage of spending on prescription medications has remained relatively unchanged since the 1960s,\(^\text{14}\) and is predicted to remain the same for the next decade.

Another way of examining drug costs is as a component of the “Nation’s Health Dollar.” According to a 2014 Centers for Medicare and Medicaid Services (CMS) report, the largest share of the health dollar went to hospital care (31 percent) followed by physicians and clinics (21 percent) and prescription medications (10 percent).\(^\text{15}\) Even more interesting is the breakdown in prescription drug expenditures: while 86 percent of drug costs were for generics and 13 percent were for branded medications, only 1 percent was for specialty medicines.\(^\text{13}\)

CMS data also shows that between 2008 and 2013, per capita spending on prescription medicines grew in the low single digits, even dropping below zero in 2010 and 2012. With the approval of new precision medicines in 2014 and 2015, drug expenditures increased but for 2016 and beyond, CMS expects growth to be in line with overall health care spending through 2024.\(^\text{16}\)
Advances in Treatment Yield Large Gains for Patients

In the debate over the cost of disease-altering medicines, a common question is the intrinsic value of novel medicines that originally demonstrate survival benefits of months when approved by FDA for the treatment of serious illnesses like late-stage or inoperable cancers.

From the standpoint of patients and their families, having the ability to spend more time with loved ones, return to work or take care of themselves, should be a paramount to society without a price tag attached. However, policymakers need real-world evidence to inform their decision-making. Therefore, researchers traditionally use post-approval studies and clinical practice experience to assess the therapy’s value over time. While a treatment’s clinical properties don’t change, a new medicine initially approved for a narrow indication may later be found to be effective in treating the disease at an earlier stage, increasing survival duration or effective in treating a different disease in combination with another medicine.17

A tyrosine kinase inhibitor (TKI) therapy, for example, was evaluated in a study conducted in 2012, which estimated the total value of survival gains associated with a first- and second-line TKI therapy, Gleevec (imatinib), in chronic myeloid leukemia (CML). Gleevec was first approved by FDA in 2001 for advanced stages of CML. It was also indicated for the use as a second-line treatment for the chronic phase of CML after failure of interferon-alpha therapy. In 2003, the therapy was approved for use as a first-line therapy. The introduction of TKI medicines and expanded use in CML has generated significant survival gains and social value. In fact, the TKI drug class in first- and second-line CML treatment has created over $143 billion in social value. Approximately 90 percent of this value derived from survival gains is retained by patients.18,19

Relative Survival Rate for Multiple Myeloma Patients Soar with New Innovative Therapies

![Graph showing relative survival rate for multiple myeloma patients with new innovative therapies.](https://example.com/myeloma_graph.png)

* Based on recent trends in the 5-year relative survival rate, for myeloma patients diagnosed in 2014, the relative overall survival rate may have reached as high as 66%.
** y-axis skips from 0 to 22 and then continues in intervals of 4 from there on
*** SCT = Autologous Stem Cell Transplant
**** IMiD = Immunomodulatory imide drugs (IMiDs) & Proteasome Inhibitors

Cost Containment Is Built Into the Drug Pricing Lifecycle

When policymakers and the public consider the cost of prescription medicines, most view branded and generic drugs differently. In fact, they represent different points in the same drug lifecycle.

At the beginning of the lifecycle, innovative biopharmaceutical companies invest in and develop novel medicines that are approved, enter the market and spend a short part of their lifespan as premium therapies. Then, the brand drug’s patents expire and the invention is passed onto generic manufacturers, who market the innovative medicines at 80 to 90 percent discounts, reaping significant value and savings to society. Today, more than 90 percent of medicines prescribed to patients are generic medicines.

Demonstrating the benefits of this system is a report from IMS Institute for Healthcare Informatics, which analyzes medicine use and spending annually. IMS concluded that between 2006 and 2014, the pricing lifecycle of innovative medicines saved the Medicare Part D program an estimated $56 billion as the share of generic medicines increased over time. In 2006, generic medicines represented 60 percent of Part D prescriptions; by 2011, the number had grown to 80 percent.20
Why Patients’ Out of Pocket Costs Have Gone Up for New Medicines

What is the reason patients are paying more for their medicines? The answer is not as simple as the prices medical innovators charge for new medicines. Rather, patient costs are determined by a number of factors, including what hospitals and health providers charge for these medicines and the extent to which insurers discriminatedely shift the costs to patients through high copays and deductibles.

Regarding the role of hospitals and physician practices, patients often pay significantly more for their medicines when the drugs are dispensed in the clinical setting. This is because manufacturers sell their medicines at a published “list” price, which hospitals and clinics routinely mark up when billing patients. As a case in point, two prominent North Carolina newspapers (the Charlotte Observer and the Raleigh News & Observer) recently reviewed the insurance claims for seven cancer therapies in the state and reported that all but one drug were more than 45 percent higher in hospitals and hospital-owned clinics. Using the cancer drug AVASTIN as the example, the analysis showed that payers were billed from $7,649 to $22,680 for the medicine, depending on where patients were treated, compared to an average sales price of $6,000 for AVASTIN.21

When it comes to the role insurance companies play, patients pay proportionately higher out of pocket costs for prescription medicines than they do for hospital and physician costs (which are sometimes negotiated), a structural leftover from the way health care worked in the earlier part of the 20th century. Applying recent CMS estimates, patients’ out-of-pocket costs on average cover 4 percent of inpatient hospital costs, 7 percent of hospital outpatient expenses, 9 percent of emergency room charges and 17 percent of their physician services. In contrast, payers now shift almost one-fourth of the costs for prescription medicines (22 percent) onto patients through high copays and insurance plans’ drug utilization review practices.22

One disturbing insurance practice is what is called “step therapy” or “fail first,” where patients are required to fail on medication after medication until the insurance company finally agrees to pay for the drug prescribed by their doctor. Equally onerous is the practice by commercial health insurers to move newer therapies into the highest “specialty tier.” This means that instead of being charged a fixed co-payment for their medicine, patients are often required to pay a percentage of the drug’s cost, which can mean thousands of dollars for a single drug that is medically necessary for patients with serious and debilitating diseases.

According to a recent Leukemia & Lymphoma Society report, even the new state exchange plans for previously uninsured patients use “step therapy” as a cost containment strategy. As a result, the nation’s most vulnerable patients can pay up to 50 percent of the costs of new cancer therapies, which leads to poor adherence.23 In fact, a 2011 study published in the Journal of Oncology Practice showed patients taking oncology medications with an out-of-pocket cost greater than $200 are at least three times more likely to choose not to fill their prescriptions than those with out-of-pocket costs of $100 or less.24

There is also the practice of shifting as much as 25 percent of the cost of oral anticancer medicines to patients in the form of higher copays25 – even though these therapies can reduce the total cost of cancer care, are less invasive than intravenous infusions, may carry fewer side effects and patients don’t have to travel to the doctor’s office every week and take time away from work or family. Yet because the copays can be hundreds or thousands of dollars per month, the American Society of Clinical Oncology (ASCO) estimates that as many as 10 percent of patients do not fill oral prescriptions due to the added cost burden, thus hampering their treatment.26

Recognizing the devastating financial impact these practices have on patients, advocacy and public health organizations are pressing Congress and state legislatures to cap co-pays on specialty medicines and ensure equality of access and insurance coverage for all treatment regimens. To date, 42 states have enacted oral chemotherapy access laws, while 15 states and the District of Columbia have either introduced or passed bills to limit what patients pay for specialty medicines.
Is Drug Pricing Really Unsustainable?

Focusing on new specialty medicines that treat cancer, hepatitis C, inflammatory diseases, multiple sclerosis and other serious diseases, insurers and others have claimed for decades that the costs of these novel therapies will bankrupt the health care system. However, the facts don’t support this contention.

According to a 2016 drug trend report, prescription medicine costs increased by just 3.8 percent, a 26.9 percent decrease from the 5.2 percent increase in 2015. What’s more, the average unit cost of prescription medicines rose only 2.5 percent, a 21.9 percent decrease compared to 2015.  

Looking specifically at spending on cancer therapies, oncology drugs – including novel medicines that stimulate the immune system to attack tumors or that target specific cancer-causing mutations – account for approximately 1 percent of the health care dollar. Furthermore, health economic studies conducted by researchers at the University of Chicago and the National Bureau of Economic Research show that the cost of new cancer medicines represent less than 1 percent of the value of the mortality reduction they yield.

Reinforcing these findings, a 2010 paper published in the Journal of Health Economics quantified the value of recent gains in cancer survival from new therapies, attributing more than four-fifths of the value created by new drugs to patients and society (81-95 percent) while less than one fifth (5-19 percent) flows to health care providers and medical innovators. Moreover, because innovative medicines extend and improve the quality of life for people with cancer, and at the same time reduce the burden on healthcare systems, the total share of spending on cancer therapies declined from 4 percent in 1995 to 3.7 percent in 2012 even as actual spending on these medicines has increased over time.  

Also of note are the realities of the health care marketplace, especially when there is competition and choice from other patented drugs and large discounts to public and private payers. Using new treatments for HCV as the example, the availability of four different novel HCV drugs has reduced prices significantly. In fact, the first company to receive approval for its novel HCV drugs, Gilead Sciences, Inc., announced in February 2015 that it planned to reduce the prices for its HCV medicines by 46 percent in the U.S. Further bringing down the costs, large payers have negotiated discounts of between 20 percent and 50 percent off the list price of the new HCV medicines and government payers have achieved even greater discounts based on mandatory and supplemental rebates and best price guarantees.
Would the U.S. Benefit from European-Style Price Controls?

In Europe, Australia and Canada, brand name drugs tend to be cheaper than in the U.S., largely because foreign governments impose stringent price and utilization controls. Moreover, countries such as the United Kingdom apply a cost-effectiveness threshold (QALYs) when assessing the “value” of all new medicines.

Using this approach, the UK’s National Institute for Health and Clinical Excellence initially restricted access to the lifesaving drug Gleevec and recently rejected coverage of a breakthrough therapy for lung cancer called crizotinib, despite evidence of substantial survival gains. However, these cost saving measures may come at a human price. According to a 2012 study in Health Affairs, U.S. cancer patients survive approximately two years longer than those in the U.K. and Europe.\(^{39}\)

What’s more, the net price for Gilead’s hepatitis C treatment, Harvoni, in the U.S. is below the medicine’s net price in the EU and Japan without the implementation of price controls in the U.S.\(^{40}\)

The Unintended Consequences of Controlling Price and Utilization

Besides delayed access to drugs and the poorer health outcomes that can result, price and utilization controls in Europe, Australia and Canada have produced significant and often overlooked unintended consequences for the economies of these regions. According to an analysis of European countries, research and drug discovery dropped precipitously since the 1990s as R&D has shifted to the U.S.

Specifically, researchers found that between 1992 and 2002, U.S. investment in R&D increased by 11 percent a year to $26 billion while European investment declined to $21 billion annually and these trends have intensified over time. The consequences are evident in a dramatic shift in the number of clinical trials now being conducted in the U.S. compared to Europe as well as fewer high value-added jobs in European countries and fewer new drug patents that translated into commercial products.\(^{41}\)

The slower rate of European investment in medical innovation has also led to fewer drug launches in Europe. According to the same analysis, from 1993 to 1997, Europe accounted for 81 unique new drugs, known as new molecular entities (NME), compared with 48 launched in the U.S. But the situation reversed over the next five years (1998-2002), when there were 44 NME launches in Europe versus 85 in the U.S. The reason this matters is because the location of new drug launches has significant bearing on how quickly doctors and patients can access the most advanced treatments. Today, the U.S. represents 5 percent of the world population and more than 80 percent of its medical innovation discoveries.
The Bottom Line: Continued Innovation Is a Cost Saver

It is true that prescription medicines come with costs. But the benefits of cutting-edge treatments – to patients, the health care system and the economy – are many times greater than their price tag.

Underscoring this fact is a recent study by the IMS Institute for Healthcare Informatics, which found that new treatment options are making patients healthier in a variety of disease areas, including oncology, hepatitis C, multiple sclerosis and diabetes. As a result, there has been a shift in spending of health care dollars, away from doctor offices visits, hospitalizations and long-term care facilities and toward new medications. In fact, reduced hospital expenditures associated with increased prescription drug coverage to millions of beneficiaries is estimated to have produced aggregate savings of approximately $1.5 billion per year, or approximately 2.2 percent of the total cost of Medicare Part D in 2011.

Also worthy of note are the broader “savings” when people live longer, more productive lives. Again using cancer as a case in point, cancer patients have enjoyed an aggregate of 23 million additional years of life and generated 1.9 trillion in additional economic activity between 1988 and 2000, because of innovative medicines. This means they have more years to spend with loved ones, to work, to consume, to pay taxes and to contribute to society.

When the costs of medicines are determined within this larger context, the real value of innovative medicines becomes clear. Medicines represent a small portion of our nation’s healthcare cost, and in fact, generate significant savings to the health system, but more importantly, allow patients to live longer, healthier and more productive lives for the benefit of their families and our larger society.


40. “Comparison of Hepatitis C Treatment Costs” IMS Institute for Health Economics Research.

