

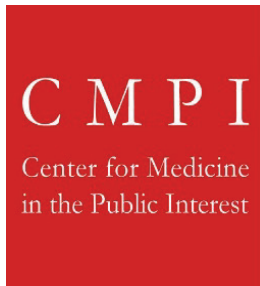


Price vs. Value:

The story and the rest of the story

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Price vs. Value: The story and the rest of the story

By Peter J. Pitts & Robert Goldberg

September 1, 2015

A new report, by Public Citizen and Carleton University, claims \$69.3 billion was spent on prescription drugs through Medicare Part D in 2013. The report points to research from Avalere Health showing roughly 58 percent of Part D spending in 2011 went to brand name manufacturers.

The report urges Congress to pass legislation allowing Medicare to reduce brand name drug prices to at least the level of Medicaid or the Veterans Health Administration and to introduce mandatory generic substitution for all plans under Part D. Currently, the federal government is prohibited from leveraging its Part D purchasing power even though private plans obtain substantial rebates from drug makers and pharmacies. And Senator Sanders has called for “wartime powers” to break the patents on the drug.

It’s time for a careful examination of the rest of the story ...

Price vs. Value: The story and the rest of the story

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Separately, more than 100 oncology doctors called for cutting the prices of cancer drugs. All new cancer drugs approved by the FDA in 2014 were priced above \$120,000 per year of use, according to their article in *Mayo Clinic Proceedings* claiming that even as the price of new cancer drugs increases, the net benefit – measured in terms of overall average survival – is marginal.

These critics make five claims:

1. That drug prices, compared to other developed countries, are much higher – true but most developed countries also spend more on medicines as part of their healthcare budget than we do in US
2. That most new drugs are little different than other medicines or, at best provide incremental value; this assumption is based on average efficacy rates rather than individual patient outcomes, as we foray into the world of precision medicine individual patients who would benefit the most will be identified as we get closer to the realizing the mantra of the right drug, to the right patient at the right time
3. That new drugs that are impactful are too expensive and therefore a) prices should be controlled or negotiated to increase access and b) generic substitution of medicines should be mandatory, allowing use of new medicines only after a generic treatment has failed to help a patient get better – most if not all private and public payers mandate generic

substitution when applicable; physicians only choose to use a brand name medicine if patient is intolerable to the generic equivalent

4. Competition is non-existent in the new expensive medicines therefore pharmaceutical companies gauge the system – not true we know that in the new Hepatitis C treatment market pharmaceutical companies are offering greater than 50% discounts to private payers; unfortunately payers are not transferring those savings to the patients.
5. Critics claim that spending on prescription drugs is unsustainable and not worth the cost. By that they mean innovator companies should not make the profits generated each year – Pharmaceuticals continue to consume only 10% of healthcare costs in the US and only 2 out of 10 medicines approved will be profitable enough for the drug companies to re-coup their investment.

Let's set aside that critics have been making the same arguments and proposing the same policies for 60 years. Their urgency is a function of ideology, not reality. Such arguments ignore the immense value the accumulation of medical innovation has had on turning the tide against disease and increasing the quality of life and economic well-being of billions of people around the world. In particular, the critics ignore the role more innovative medicines make in reducing the cost of medical care, reducing the pain and suffering of sick people, maintaining the ability of people to remain in the workforce, pay taxes and spend money. And they ignore the fact that a limit on return on investment -- through price controls, patent seizures, etc., reduces the number of new medicines (and their generic imitators) and limits on access to such innovations increases the cost of health care and rate of death.

In the last 100 years, life expectancy in US has increased based on three simple interventions, clean water or sanitation; better surgical and diagnostics and most importantly innovative biopharmaceuticals

Today we are more obese, more sedentary, more stressed, we exercise less and eat poorly – but somehow we live longer than 10, 20 or 30 years ago.

NOW, THE REST OF THE STORY: VALUE

Let's start with Sovaldi. Solvadi, the new Gilead medicine that cures the two major genotypes of Hepatitis C has become the poster child for critics. The Public Citizen and Carleton University do not mention in their report that one pre-Sovaldi “best

practice” treatment for Hepatitis C, the drug Pegasys, requires one injection a week for 48 weeks — and very few patients see the treatment through to completion, so much of that treatment, both physician time and drug cost, is wasted. Nor is it that much cheaper: At about \$7,000/month, the full course of treatment is over \$70,000 — barely less than cost of the three months needed for Sovaldi to work a cure. You forget that the “complaining physicians” make more money on injections than by prescribing a pill that you swallow. And Sovaldi is the poster child of precision medicine, if you don’t have that phenotype you don’t get the prescription.

Critics of the price of new Hep C drugs claim that their adoption would cost \$240 billion and raise premiums about \$1500 over five years. First, these arguments ignore what the cost of treating HCV patients with current approaches. For instance, the total lifetime cost of treating every HCV patient absent such innovations would be \$360 billion. That does not include an estimated \$400 billion loss in productivity and \$3 trillion in health value lost because of premature mortality.

From this perspective it is clear that new medicines almost always reduce the cost of living longer and healthier life and increase the value of such improvements. Further, it is clear that the price of new drugs, setting aside the obvious need for faster and smarter drug development costs, reflect the high percentage of social value generated by medical innovation. Assuming the \$240 billion cost goes right to innovators, more than 90% of the value of the product (\$360 bill + \$400 bill. + \$3 trillion = \$3.76 trillion) goes to society.ⁱ Two recent studies also confirmed the long term value of using such medicines.ⁱⁱ

And the price of not using Sovaldi is very high. One in three patients with the Hepatitis C virus eventually develops liver cirrhosis, and managing these patients is costly. A “routine” liver transplant (where the liver is from a cadaver) costs close to \$300,000; a “living donor” transplant is even more expensive. But why let the facts get in the way.

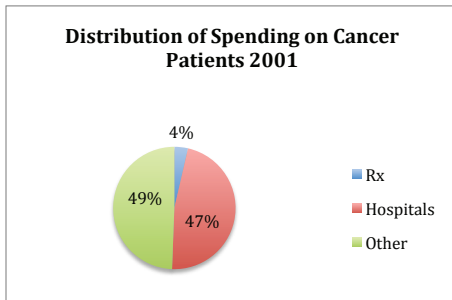
Data recently published by the PwC Health Research Institute proves the reverse. The study shows that the use of Sovaldi will actually drive down overall spending within a decade.

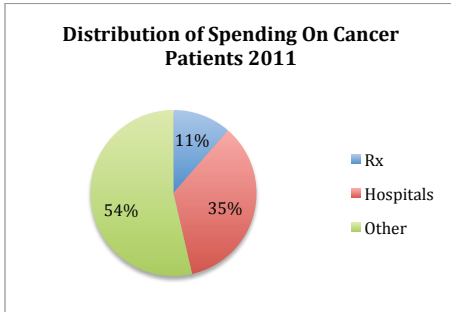
Also, is anyone really paying “\$1000 per pill?” Certainly nobody with insurance. And for those without coverage there are generous programs supplied by the manufacturer. Large payers negotiated discounts of between 20-50 % off of the list price but these discounts were not passed on to the consumer. Government payers get even more based on mandatory rebates, supplemental rebates and best price guarantees and protections to any price increases; these include Medicaid and VA patients.

New Medicines Actually Reduce The Treatment Costs Of Cancer and Other Diseases

As the number of people using new medicines increases, their health care costs as a percent of total health care spending has declined.

In 2011, 11.4 percent of total cancer expenditures were for prescription medicines, as compared with 3.6 percent in 2001. The proportion spent on inpatient hospital stays declined from 47 percent in 2001 to 35 percent in 2011.





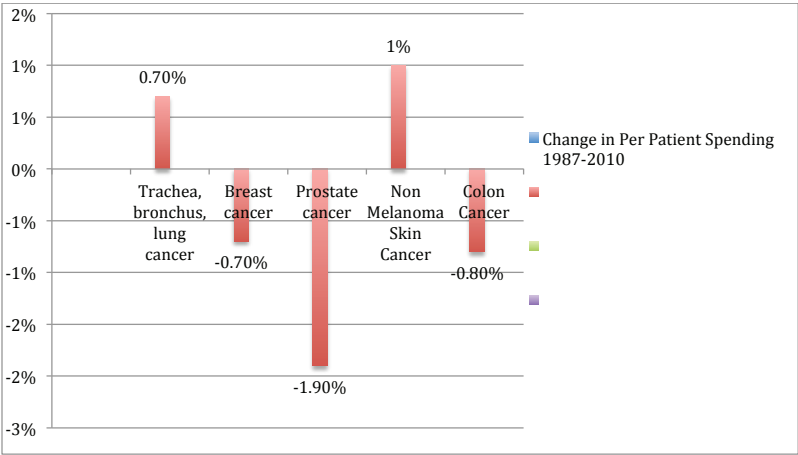
Source: AHRQ, Trends in Use and Expenditures for Cancer Treatment among Adults 18 and Older, U.S. Civilian Noninstitutionalized Population, 2001 and 2011.

During that time the per capita cost of treatment in current dollars remained the same at about \$5600. ⁱⁱⁱ

In real dollars the average growth rate in per patient spending on cancer care actually **declined as the chart below shows –because we are hospitalizing less frequently:**

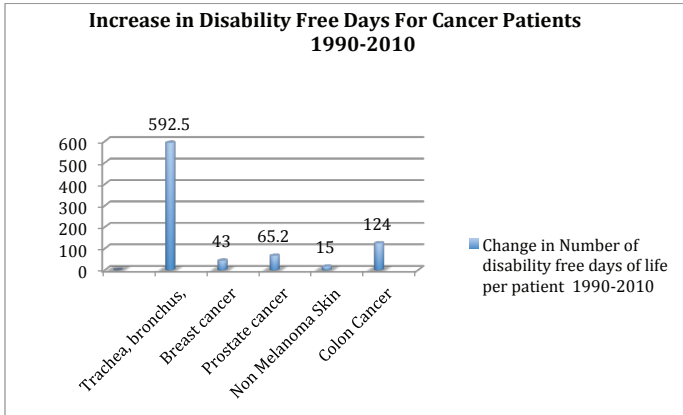
Cost of Cancer Care Declines

1987-2010



Source: Bureau of Economic Analysis, Department of Commerce 2015

In fact, “the net value of treatment has grown substantially, consistent with medical technology improving over time and leading to better health outcomes at a lower cost per patient.”^{iv} From 2000-2010 20 of the 30 chronic diseases studied experienced an increase in health outcomes over the period. Every cancer studied (trachea, bronchus, lung, prostate, colon, breast, and melanoma) showed greater increases in health outcomes over time in terms of additional days of disability free life.

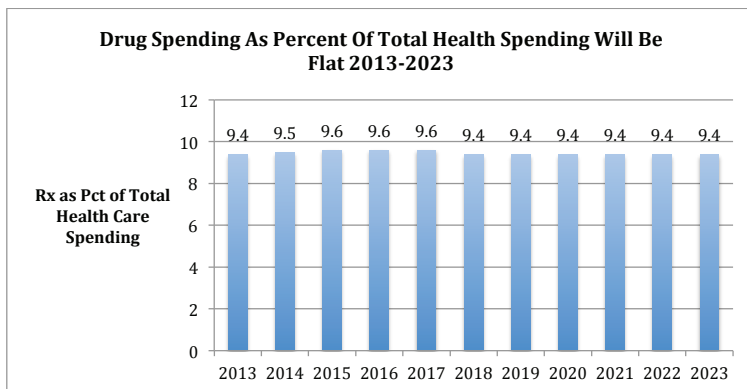


Source: Bureau of Economic Analysis, Department of Commerce 2015

Is Drug Spending Really 'Unsustainable'?

Perhaps future drug spending on new medicines, expressed as a percentage of total spending will be “unsustainable” and therefore require PBMs and insurers to increase out of pocket spending by the sickest patients.

In fact, the share spent on medicines will remain flat for the rest of the decade.



Source: Centers for Medicare and Medicaid Services

Media Understates and Hides Increase in Out of Pocket Drug Costs

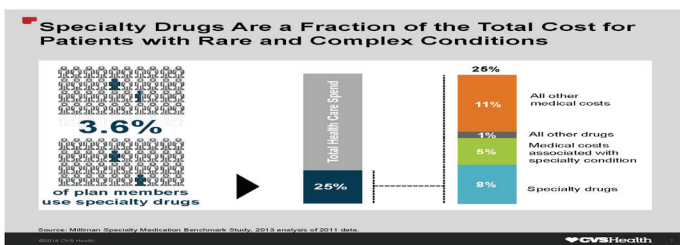
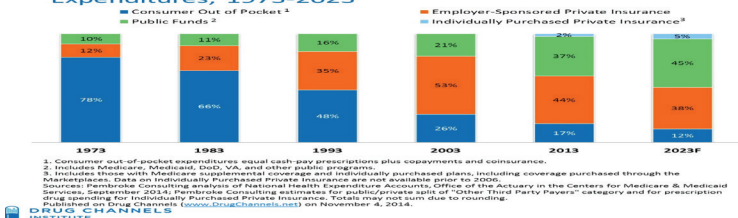
Media reports repeated the claim made by Express Scripts that patients who used \$50000 or more of medicines paid from \$1773 - \$2782 out of pocket in 2014. This estimate also includes Medicare patients who pay only 5 percent of the cost of a drug after an out of pocket maximum is met, and Medicaid patients who have a nominal co-pay of \$3-5 for all medicines. Thus, the Express Scripts study masks the burden on consumers in health exchange plans.

Moreover, HHS estimates that out of pocket spending for drugs, as percent of total drug expenditures, will actually increase 27 percent over the next decade.

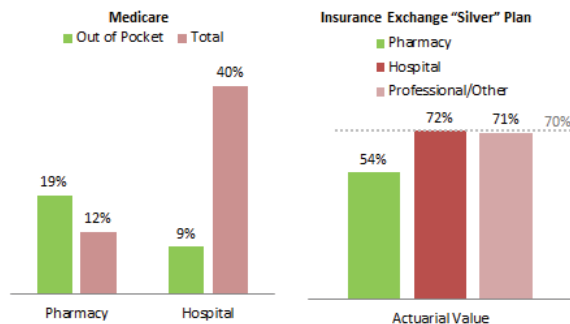
This shift began a decade ago when PBMs and insurers created formulary tiers of increasingly higher cost and placed newer medicines for chronic conditions on the highest cost-sharing tier.

The truth is that out-of-pocket (OOP) expenses for most patients are lower now than a decade ago.

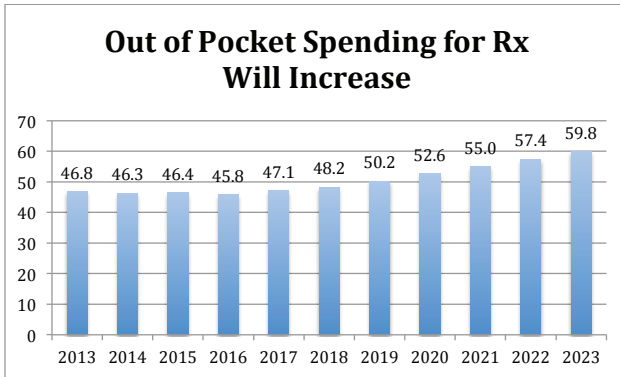
Sources of Payment for Outpatient Prescription Drug Expenditures, 1973-2023



Formulary Cost Sharing Higher for Medicine



Impact of Health Insurance Marketplace on Participant Cost Sharing for Pharmacy Benefits, Milliman May 2014
National Health Expenditures 2014 and KFF Jul 2014, How much is enough Out of Pocket Spending for Medicare Beneficiaries

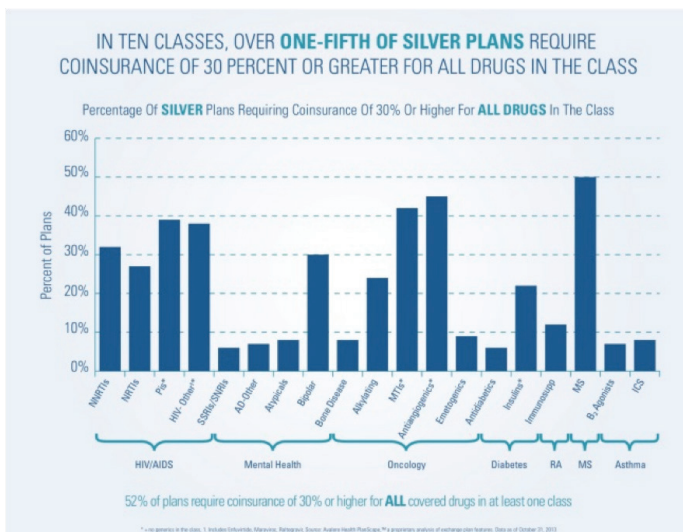


Source: Centers for Medicare and Medicaid Services

But studies have shown that patients stop taking medicines when out of pocket costs increase beyond \$200. Nearly 1 in 4 patients stop taking medicines when their share is \$200, and a greater number stop when the cost climbs beyond that.

Additionally, when out-of-pocket costs reached \$2,000 or more, the number of members abandoning their new prescription was nearly 19-24 times higher compared to members with out-of-pocket costs less than \$100. ^v

Despite overwhelming evidence that higher cost-sharing reduced use of new medicines and leads to higher treatment costs, PBMs and health plans have placed drugs for cancer, rheumatoid arthritis, and multiple sclerosis in the highest cost-sharing tier. The number of Silver plans in the health exchange that charge patients 30 percent or more has increased to 41% in 2015 from just 27% in 2014.



Such practices have been singled out as violating the civil rights of patients. As the Department of Health & Human Services has stated: “placing most or all drugs that treat a specific condition on the highest cost tiers discourages enrollment by individuals based on age or based on health conditions, in effect (is) making those plan designs discriminatory.”^{vi}

Most media coverage of the cost of prescription drugs ignores important information about the relative amount of money spent on new medicines for the most expensive patients. Not only are new medicines a very small portion of total health care spending, the use of new therapies is associated with a decline in the use of other medicines, hospitals, and physicians.

All told, over the next decade, as more new medicines come to market, drugs will remain a relatively small but increasingly important portion of what we spend on health care. At the same time, PBMs like Express Scripts have been given control

over what medicines people will use and how much they can pay for them, though they have no direct role in treating people. Nor are they accountable for their health.

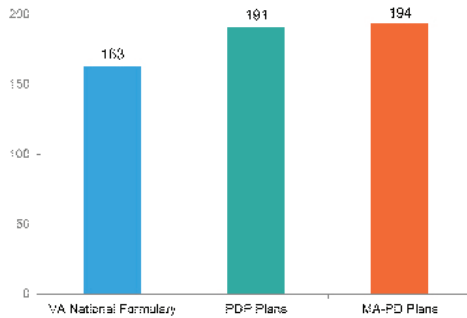
Rather, PBMs have used this control to maximize profits. Specifically, they have cooperated with health plans to ensure that most or all medicines to treat the small percentage of patients with the highest costs are more expensive and more difficult to obtain. In doing so, they have increased the risk that people with such conditions will get sicker or die.

For example, prior to the introduction of the HIV drugs in 1996, HIV positive individuals had no means of buying a longer and healthier life. Ditto stomach cancer patients prior to Sutent and new drugs for Hepatitis C.

Absent those innovations, longer life could not be bought anywhere at any price. Thus, the introduction of such innovations actually leads to a dramatic reduction in the cost of a longer life. In fact, the long-term decline in the rate of health care spending is a function of a dramatic shift in treatment settings for cancer, heart disease, stroke, hip replacements, etc. made possible by new medicines.

Let's tackle the VA next. The Veterans Administration's national formulary does not offer access to nearly one-fifth of the top 200 most commonly prescribed Medicare Part D drugs. Among the 37 drugs not covered by the VA, PDPs cover 19 medications at least 90 percent of the time. The vast majority of these 19 medicines predominately fall on either the generic tier or the preferred brand tier of PDP plans.

AVERAGE COVERAGE OF TOP 200 MEDICARE PART D DRUGS
BY VA NATIONAL FORMULARY, PDPs, AND MA-PDs (2015)



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And a study from Columbia University found that just 19 percent of all new drugs approved since 2000 were covered by the VA and just 38 percent since 1990. Media reporting missed these facts too. For a more detailed examination of the VA program, see Appendix A.

Per “negotiating prices” for Medicare Part D, allowing the Federal government to negotiate drug prices would result in prices going up and patient choice going down. That’s why the Non-Interference Clause, the legislation that prohibits Federal price negotiation was created in the first place. It’s interesting and important to note that the legislative language was drafted by Senators Ted Kennedy and Tom Daschle.

The Congressional Budget Office found that between 2004 and 2013, Part D cost an extraordinary 45 percent less than what was initially estimated and premiums for the program are roughly half of the government’s original projections. These unprecedented results are largely due to Part D’s market-based structure. Beneficiaries are free to choose from a slate of private drug coverage plans, forcing

insurers to compete to offer the best options to American seniors. It's hardly surprising that the program has led to low prices and satisfied customers. Through their own negotiations with drug makers, private insurance plans that operate under Part D have already had great success in keeping pharmaceutical prices down. In fact, the CBO has observed that Part D plans have "secured rebates somewhat larger than the average rebates observed in commercial health plans." What's more, the CBO has said that doing away with the non-interference clause "would have a negligible effect on federal spending." In a report from 2009, they reiterated this view, explaining that such a reform would "have little, if any, effect on [drug] prices." In fact, allowing the feds to negotiate drug prices under Part D would likely have a negative effect on the program. The CBO predicts that when HHS forces pharmaceutical firms to lower the cost of a particular drug, this tactic brings with it "the threat of not allowing that drug to be prescribed."

The critics have been led by Dr. Peter Bach who has hammered home the claim that notes the average launch price of cancer drugs has climbed \$8,500 per year from 1995 to 2013.^{vii} But during that time cancer survivorship surged from 10 million to 14 million people and life span expressed by 36 million life years worth about \$3 trillion.^{viii}

Back assert spending on new cancer drugs bankrupts our healthcare system. But the benefits to patients are palpable. Drugs that emancipate our immune system to attack tumors or target specific genetic cancer causing mutations have transformed cancer care. These cancer drugs are expensive no doubt. Yet they account for only account for 0.7 percent of the \$2.9 trillion we spend on health care. ^{ix} Cancer spending has increased in 1995 from \$42 billion to about \$130 billion today. But its share of total health spending declined from 4.7 percent to 4.4 percent during the same time period.^x

Bach has proposed restrictive drug formularies and price controls to reduce new drug prices and claims it would be "superior to the situation we have today".^{xi}

He asserts that unless we limit access to new medicines high prices would “get passed on “through individual costs or insurance.”^{xii} Yet two studies concluded removing such restrictions only raises premiums about a dollar a month.^{xiii}

Bach would price the same treatments different based on average survival for different conditions. He’d pay \$10320 a month using Erbitux for early stage head and neck cancer because it adds 16 months of life and \$470 a month in advanced head and neck tumors because it adds ‘only’ two months of life. He dismisses that as Erbitux’ “least effective use” even though it was the first treatment in 20 years to extend life in metastatic head and neck cancer.^{xiv} Bach’s pricing scheme would reinforce fail first practices and rob patients of progress against incurable diseases. The first anti-AIDS treatments added ‘only’ two months of life in 1987. By 2000 a series of new drugs added 15 years of life. We’d have forfeited such advances under his scheme. (And where’s the evidence that Sloane Kettering should charge more than MD Anderson or any cancer center or versus Kaiser?)

And as far as Senator Sanders’ call for “wartime powers to break patents,” there is no such thing as a free lunch – let alone “free” innovation. While opaque and seemingly arbitrary drug pricing deserves immediate attention, the value of innovation must not be ignored. Innovation is hard. Today it takes about 10,000 new molecules to produce one FDA-approved medicine. This observation itself is disconcerting, but, further, only 3 out of 10 new medicines earn back their R&D costs. Moreover, unlike other R&D-intensive industries, biopharmaceutical investments generally must be sustained for over two decades before the few that make it can generate any profit. Innovation is slow. As any medical scientist will tell you, there are few “Eureka!” moments in health research. Progress comes step by step, one incremental innovation at a time. Indeed, empirical research has demonstrated that patent protection is critical to reducing the uncertainty that characterizes the drug development process. And breaking patents would simply cause a shift in investment overseas or to other industries where patent protection is stronger or development cycle times are shorter and less predictable. ^{xv}^{xvi}

As Abraham Lincoln said, “Patents add the fuel of interest to the passion of genius.”

But don't all these wonderful innovations come from government-funded research? Nope. A study in Health Affairs by [Bhaven N. Sampat](#) and [Frank R. Lichtenberg](#) (*What Are The Respective Roles Of The Public And Private Sectors In Pharmaceutical Innovation?*) <http://content.healthaffairs.org/content/30/2/332.full.html> puts the issue in a data-driven perspective that gives the NIH its due – but in the proper frame of reference.

For example, according to Sampat and Lichtenberg, fewer than 10 percent of drugs had a public sector patent, and drugs with public-sector patents accounted for 2.5 percent of sales, but that the indirect impact was higher for drugs granted priority review by the FDA. (Priority review is “given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists.)

478 drugs in our sample were associated with \$132.7 billion in prescription drug sales in 2006. Drugs with public-sector patents accounted for 2.5 percent of these sales, while drugs whose applications cited federally funded research and development or government publications accounted for 27 percent.

And as far as “mandatory generic substitution” is concerned, from a therapeutic perspective, generic substitution, while a good way to save payers money in the short term (including our nation’s largest payer—Uncle Sam), often has quite deleterious impact on patient care—an issue that is well researched and quantified. And, this is even more important when it comes to medicines that have a narrow therapeutic index.

According to the FDA, narrow therapeutic index means that “small changes in blood concentration have the potential to result in serious therapeutic failures and/or serious adverse drug reactions.”

(Currently, the “sameness” of a brand product and a generic version is evaluated based on two-treatment crossover study to prove bioequivalence, the aim being to

show that the 90 percent confidence intervals of the geometric mean test/reference ratios for both maximum plasma concentration and the area under the plasma concentration-time curve fall within a range of 80 percent to 125 percent.)

In fact, this is such an important issue, that at recent meeting of the Generic Candidate Pharmaceutical Association, CDER Director Janet Woodcock said that the FDA is discussing tightening the equivalence limits of generic medicines “so there is less variability.” And the agency’s Pharmaceutical Science and Clinical Pharmacology Advisory Committee concluded (in an 11-2 vote) that current bioequivalence standards are not sufficient for narrow therapeutic index drugs.

A study fielded by the National Consumers League demonstrated that switching a patient to a generic medicine doesn’t always result in positive outcomes:

- 15% of general Rx users saying that they or a family member experienced therapeutic substitution.
- Nearly half of Rx users (47%) were dissatisfied (or their family was) with how the process occurred and report that this substitution did not result in lower out-of-pocket costs.
- More than a third (40%) said that the new medication was not as effective as the original one, and nearly a third (30%) experienced more side-effects following the substitution.
- Large majorities of Rx users think that the potential side effects of the new medication, the patient’s medical history, how well the drug works and the prescribing physician’s opinion are factors that are ***absolutely essential*** when decisions are made about therapeutic substitution.

The repercussions of choosing short-term savings over long-term results, of cost-based choices over patient-centric care, of “fail first” policies over the right treatment for the right patient at the right time – are pernicious to both the public purse and the public health. Skimping on a more expensive medicine today but paying for an avoidable hospital stay later is a fool’s errand.^{xvii} Several studies have shown that step therapy, with mandated generic substitution, does not save money; it makes people sicker and increases the cost of care.^{xviiiix}

Alas, Sanders never mentions the very real effect such a policy would have on patient care. It's just soundbite politics courtesy of the Senator from Ben & Jerry's.

As John Adams said, "Facts are pesky things." Facts that don't reinforce your cognitive mapping are pesky things. And nowhere is that more of an issue than in the debate over drug pricing.

Price Controls

A new [study from the Institute for Health Policy](#) compares drug prices in four countries and points out that countries such as Germany, the United Kingdom and Australia, have governmental regulations that help keep drug prices at "sustainable levels." The study authors urge policymakers to "do something" to change the situation in the U.S.

The new paper is full of statistics. But, as the saying goes, statistics are like a bikini – what they show you is interesting, but what they conceal is essential.

In Europe, Australia, and Canada, brand name pharmaceuticals tend to be significantly cheaper than they are in the States -- largely because foreign governments impose stringent price controls on most drug sales. (The study conveniently neglects to mention that generic drugs are more expensive in Europe than in the US. And generic drugs represent 87% of all the drugs sold here at home.)

When it comes to new, innovative medicines for many serious and life-threatening diseases, government-dictated lower prices come with a very high price tag. Free health care like in Europe and Canada? Let's look at the record. Government controlled health care is not free. It comes at great cost through higher taxes, wait times, and denials of coverage. According to the OECD, the French pay about 20% more in income tax while Canadians, according to the Frazier institute, wait an average of almost 18 weeks from a general practitioner's referral to treatment by a specialist. In Canada about 22% of taxes go to the health system and several provinces, including Quebec, Ontario, Alberta, and British Columbia (BC) also charge additional premiums.

Citizens in the U.K. pay 11% of each pound they make in weekly income, between £100 and £670 for the National Health Service (NHS), plus an additional 1% of income over £670 a week, and though the copay for drugs is low, many drugs are not covered, often because they're not considered cost efficient. What can't be overlooked (and is conveniently absent from the Institute for Health Policy study) is that price controls equals choice controls. And choices (or the lack thereof) have consequences.

Consider the facts:

- The five-year survival rate for early-diagnosed breast cancer patients in England is just 78 percent, compared to 98 percent in the U.S.
- A typical Canadian seeking surgical or other therapeutic treatment had to wait 18.3 weeks in 2007, an all-time high, according to The Fraser Institute.
- The average wait time for bypass surgery in New York is 17 days compared to 72 days in the Netherlands and 59 days in Sweden.
- More than half of Canadian adults (56 percent) sought routine or ongoing care in 2005. Of these, one in six said they have trouble getting routine care.
- Eighty-five percent of doctors in Canada agree private insurance for health services already covered under Medicare would result in shorter wait times.
- Approximately 875,000 Canadians are on waiting lists for medical treatment.

Now consider the Affordable Care Act. Patients can access any medicine they need -- as long as it's on the exchange formulary. Sure, the ACA limits the degree to which insurers can charge higher premiums for sicker patients, but ObamaCare plans found a way around these rules: impose higher out-of-pocket costs for all or most specialty drugs. High co-pays effectively remove choice from the system for many patients.

The breakdown of Silver plans (the most popular category) is particularly revealing. In seven classes of drugs for conditions from cancer to bipolar disorder, more than a fifth of these plans require patients to shoulder 40 percent of the medicine's cost. And 60 percent of Silver plans place all drugs for illnesses like multiple sclerosis and rheumatoid arthritis in the "formulary tier" with the highest level of cost-sharing.

Nearly every Silver plan across the country, in fact, puts at least one class of drug exclusively in the top cost-sharing tier. In effect, this leaves patients with a given condition — whether HIV or Crohn’s disease — without a single affordable treatment option. Silver is the new Black.

If we’re going to look to other healthcare models for solutions, we must uncover and study their problems. Healthcare is too important to allow reform by sound bite. “Drugs from Canada” is as much a false promise as “free” healthcare.

To maximize profits, Express Scripts and health insurers are systematically placing most or all drugs that treat these and other conditions on the highest cost tiers to discourage enrollment by individuals based on age or based on health conditions. HHS notes such practices make those plan designs discriminatory.

Moreover, they are now demanding doctors roll back patient guidelines because they don’t want to take on the upfront cost of the highly effective drugs they once insisted upon. (You see, me-too drugs are a good thing.)

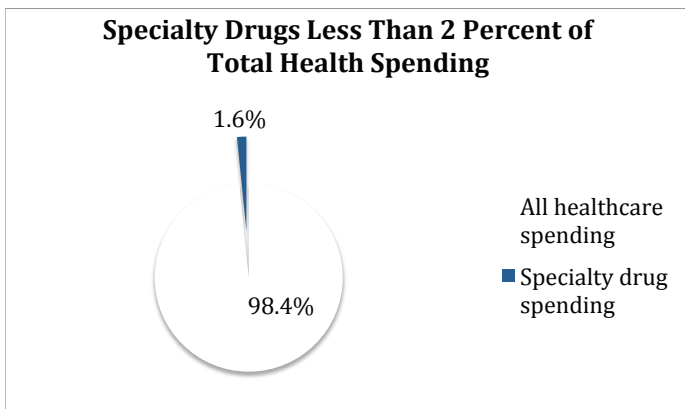
The problem is not the price of the medicine per se (and we will discuss how to drive down the cost of drug development later). Rather, as Tomas Phillipson has pointed out:

Health care insurance ensures access to health care; stated another way, this insurance provides access to medical innovations already developed. It is the innovations in treatment over the past century that partly protect us against the loss of actual health when disease hits. Medical innovation, therefore, is the key to true “health insurance.” Whereas health care insurance reduces the risk of financial shocks by pooling financial risk at a single point in time, medical innovation is the primary method by which the future risk of losses in health itself is reduced over time, and can thus be viewed as serving the role of insuring future health.

In essence, medical innovation reduces the true price of health. Innovation in treatments for breast cancer provides a salient example. Before these innovations, the price of living longer for a breast cancer patient was infinite. Regardless of

financial means or health care coverage, no one could buy a longer life. Innovations enabled breast cancer patients to buy longer, healthier lives in the presence of the disease, with access to these innovations often the result of health care coverage. Innovations in HIV treatment are another, similar, example. Thus, in the most literal sense of the phrase, cumulative medical innovation provides real “health insurance,” and investment in medical R&D is the premium required for improved risk reduction in health.

Using IMS data we find that the drug costs of super spenders is a little over 7 percent of the total spending of the 1 percent most costly cohort of patients.



Source: IMS Health Institute Private Health Plans 65 and Under.

PBMs and health plans justify placing all specialty medicines (the newest treatments for diseases) into the highest cost sharing tier and requiring patients to fail first on a series of other medicines prior to being covered for a new drug, by claiming that prescription spending is unsustainable and can make health insurance itself unaffordable.

But it's also a way of under valuing the introduction of new medicines. It is interesting that the group's demands for price controls rests on the argument of one paper written by David Howard, Peter Bach, Ernst Berndt and Rena Conti.

That because the methodology used by the authors to measure value – median survival time -- is precisely the metric that Prof Berndt demonstrates will discourage the introduction of targeted medicines. And the policies they propose not only discourage innovation, they are the exact opposite of what Berndt has proposed to improve the economics of precision medicine.

The authors claim prices should fall as the introduction of new medicines for the same type of cancer increase. This is a naïve application of basic microeconomic theory. It is more accurate to suggest that each new medicine that targets a specific

group of patients or a particular genetic mutation has its own market. The authors call this, incorrectly, a monopoly.

Recently oncologists such as Drs. Saltz and Bach – have stoked media attention by denouncing cancer drug prices– some exceeding \$100,000 a year – as immoral. They claim such prices don't reflect the treatment's value and make the cost of treating cancer unsustainable.

We'd like lower prices too, but hope to achieve reductions mostly by reducing the time and cost needed to get treatments to patients. We also believe such prices reflect the contribution new medicines make to saving lives and reducing overall treatment costs. Bach notes the average launch price of cancer drugs has climbed \$8,500 per year from 1995 to 2013.^{xx} But during that time cancer survivorship surged from 10 million to 14 million people and life span expressed by 36 million life years worth about \$3 trillion.^{xxi}

They assert spending on new cancer drugs bankrupts our healthcare system. But the benefits to patients are palpable. Drugs that emancipate our immune system to attack tumors or target specific genetic cancer causing mutations have transformed cancer care. These cancer drugs are expensive no doubt. Yet they account for only account for 0.7 percent of the \$2.9 trillion we spend on health care. ^{xxii} Cancer spending has increased in 1995 from \$42 billion to about \$130 billion today. But its share of total health spending declined from 4.7 percent to 4.4 percent during the same time period.^{xxiii}

New medicines reduce the cost incurred by a cancer diagnosis, for instance in part by reducing hospitalization. In 1996 drugs were 3.7 percent of cancer spending and 62.4 percent went to hospitalization. By 2012, drug spending was 9.3 percent of cancer costs while the share going to hospital stays dropped to 41.3 percent. ^{xxiv} If we were allocating the same proportion of money to hospitals today, as we were in 1996, we'd be spending about \$18 billion more a year on cancer. And we have yet to see the full benefit of the cancer drugs not yet included in these estimates.

As the price and number of new treatments increases, their value increases too. A recent Bureau of Economic Analysis study found between 2000-2010 that “medical technology (for treating cancer and other costly illnesses) is improving over time, leading to better health outcomes at a lower cost per patient.”^{xxv}

Moreover, patients with the most incurable cancers gain the most from new medicines. Recently studies have found treating people with advanced melanoma a combination of two immunotherapies (nivolumab and ipilimumab) can dramatically alter the prognosis of these patients. At the best-responding dose level nearly 90 percent of patients survived two years compared to 15% for patients treated with conventional chemotherapy. The treatments stopped or shrunk tumors in 58% of patients for about a year.

Saltz predicts the combo would cost \$300,000 and that giving the 589,000 people who die from cancer annually such breakthroughs would cost \$174 billion each year.

That’s a bargain.

A one-year delay in tumor progress would eliminate up to \$60 billion worth of surgery, therapy and other medicines.^{xxvi} Most important, the cumulative value of over half of million people (assuming 1 year of life is worth \$150,000) living 2 years is about \$176 billion.

Indeed, many critics focus only on the incremental cost of a new drug on generating the incremental, average survival benefit. They ignore the cumulative benefit of survival (let alone the important knowledge generated by using new and more precise treatments). Another example of great progress is the treatment of blood cancers. In 1998 myeloma patients had a median survival of 3 years. Now myeloma patients live much longer, some exceeding 10 years and being cured, mostly because of the FDA approval of 6 new medications. Over the next several years an additional 4 or more drug approval could occur. During the same time span hospitalizations, transfusion and other medical services for myeloma dropped 83 percent.^{xxvii}

Bach has proposed restrictive drug formularies and price controls to reduce new drug prices and claims it would be “superior to the situation we have today”.^{xxviii}

He asserts that unless we limit access to new medicines high prices would “get passed on “through individual costs or insurance.”^{xxix} Yet two studies concluded removing such restrictions only raises premiums about a dollar a month.^{xxx}

Bach would price the same treatments different based on average survival for different conditions. He’d pay \$10320 a month using Erbitux for early stage head and neck cancer because it adds 16 months of life and \$470 a month in advanced head and neck tumors because it adds ‘only’ two months of life. He dismisses that as Erbitux’ “least effective use” even though it was the first treatment in 20 years to extend life in metastatic head and neck cancer.^{xxxi} Bach’s pricing scheme would reinforce fail first practices and rob patients of progress against incurable diseases. The first anti-AIDS treatments added ‘only’ two months of life in 1987. By 2000 a series of new drugs added 15 years of life.^{xxxii} We’d have forfeited such advances under his scheme.

Euromania^{xxxiii}

Ultimately, critics opt for the kind of price controls applied to new medicines in Europe. They ignore two important points:

First that by adjusting our spending to EU levels through changes to drug prices alone, we’d save .33 percent of the total invested in cancer care.

Second, reducing drug expenditures by applying the same approach as EU health systems do would reduce and delay access to new medicines.^{xxxiv}

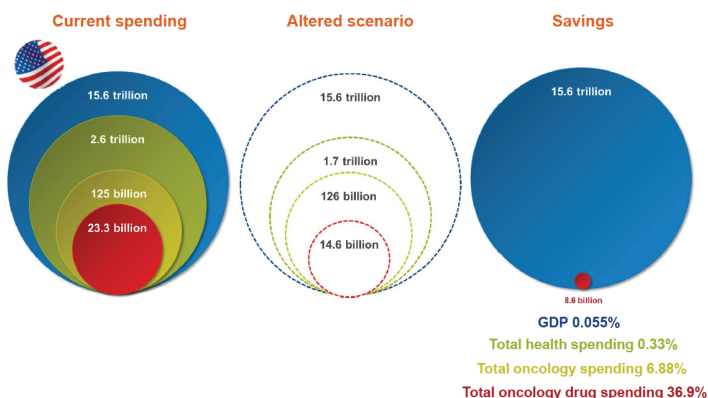
Third, this delay is directly associated with more Europeans dying from cancer and living shorter lives.^{xxxv} As a recent study of European cancer spending and mortality concluded:

“compared to low-spending health systems, high-spending systems had consistently lower cancer mortality in the period 1995–2007. Similarly, we found that the

countries that increased spending the most had a 17 percent decrease in amenable mortality, compared to 8 percent in the countries with the lowest growth in cancer spending. For excess mortality, the corresponding decreases were 13 percent and 9 percent. Additionally, the rate of decrease for the countries with the highest spending growth was faster than the all-country trend. “

Some point to Switzerland as the best replacement for the US health system: It’s essentially a government-financed program to cover the cost of private insurance. It also sets drug prices. And the results are sobering;

European spending patterns for oncology drugs in the US would save \$8.6 billion – <0.33% of the total US health spending



Calculated with US GDP and EU spending ratios from prior slides
 © GfK 2013



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Estimates of the cost per life-year gained before ages 75 and 65 in 2012 from drugs registered during 1990-2007 are \$21,228 and \$28,673, respectively. These figures are below even the lowest estimates from the value-of-life literature of the value of a

quality-adjusted life-year. The estimates indicate that the cost per life-year before age 75 gained from drugs registered during earlier periods (1985-2002 and 1980-1997) were considerably lower: \$5299 and \$3218, respectively.

The largest reductions in premature mortality occur at least a decade after drugs are registered, when their utilization increases significantly. This suggests that, if Switzerland is to obtain substantial additional reductions in premature cancer mortality in the future (a decade or more from now) at a modest cost, pharmaceutical innovation (registration of new drugs) is needed today.

Sustainable Innovation

The way to make medicines less expensive is to reduce the cost of development. Indeed, since the Critical Path Initiative was launched in 2004 (the authors were honored to have been there at the birth) the principal goal of regulatory science was to reducing cycle times for all products, particularly those where unmet need is greatest. The hope then was that personalized medicine and biomarkers would make it easier and quicker to identify smaller groups of patients that would get the most benefit and least risks from targeted treatments. Unfortunately, it often costs as much or more to develop a new drug for a smaller population than it does for a one size fits all medicine.

A recent review of the growth in the number of cancer drugs targeting specific mutations or disease mechanisms underscores how the one size fits all approach to measuring drugs using randomized controlled trials – the benchmark the critics use to evaluate clinical benefit – is rigged to discourage innovation: “the resulting segmentation of cancer into ever smaller population sub-types may eventually discourage future development of additional therapeutics as recent declines in early stage candidates may presage. Without changes in the procedures by which we develop, evaluate, disseminate and finance new medicines, even when science generates promising intervention hypotheses, if the patient subpopulation falls below a certain threshold—whether a few thousand or a few hundred—it may prove clinically impractical and economically infeasible for developers to create the

efficacy, safety and clinical benefit evidentiary data package required at drug prices payers can afford. To the extent science and clinical experience suggests combination treatments are preferable to mono-treatments, the clinical evidence generation and economic feasibility challenges are potentially even more daunting.”^{xxxvi}

Another paper by the same authors looked at the ‘uneconomic economics of personalized medicine. It concluded:” Even small population oncology indications can be made economically viable (substantial net present value) if a mix of the incentives can: Halve the development cost; Cut development time by 2–3 years; – Ensure near exclusivity (high market share). Higher pricing may also be necessary, recognizing the difficulties this places on financially challenged payers, particularly if combination treatment is required for best efficacy.”^{xxxvii}

But you won’t hear that from the critics even though it’s empirically established. While everyone is entitled to their own opinions, they are not entitled to their own facts. Unfortunately, peer reviewed medical journals publish articles claiming that drug prices are rising without regard to value or cost. This is pure fiction.

The Institute for Health Policy calls for policy makers to “do something” – and amen to that. What that something should be, however, isn’t to copy foreign schemes that trade transitory short-term cost savings for long-term patient care; it’s to support the continued innovation that saves lives and money – big money.

Drugs aren’t the cause of rising health-care costs — they’re the solution. Demonizing new treatments distracts from the real problem in US healthcare: top-down cost-centric policies that focus on the near-term, short-changing long-term patient outcomes, endangering “sustainable innovation” by denying fair reimbursement for high-risk investment in R&D. (Research and development costs big even if a drug never makes it to market — and most don’t.) New treatments are a bargain. Disease is always much more costly. If we don’t reward risk-taking on behalf of human health, both will shrink.

Referring to the Model T, Henry Ford famously said, "Any customer can have a car painted any color that he wants so long as it is black." That worked out fine – until there was competition. Choice is the great emancipator. The same is true when it comes to healthcare – and a lot more important.

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ⁱ Medical Breakthroughs And Credit Markets By Tomas J. Philipson and Andrew C. von Eschenbach. <http://www.forbes.com/sites/tomasphilipson/2014/07/09/medical-breakthroughs-and-credit-markets/> Accessed 7/25/14.

ⁱⁱ Najafzadeh M, Andersson K, Shrank WH, Krumme AA, Matlin OS, Brennan T, Avorn J, Choudhry NK. [Cost-effectiveness of novel regimens for the treatment of hepatitis C virus.](#) *Annals of Internal Medicine* 2015;162:407-19.

ⁱⁱⁱ Ibid

^{iv} [Using Disability Adjusted Life Years to Value the Treatment of Thirty Chronic Conditions in the U.S. from 1987-2010](#) Tina Highfill and Elizabeth Bernstein - BEA Working Paper (WP2014-9)

^v **AMCP: Patients more likely to start specialty meds when costs stay under \$250**
<http://bit.ly/1G85vTC>

^{vi} <http://healthaffairs.org/blog/2015/02/22/implementing-health-reform-2016-benefit-and-payment-final-rule-consumer-provisions/>

^{vii} Howard, David H., Peter B. Bach, Ernst R. Berndt, and Rena M. Conti. 2015. "Pricing in the Market for Anticancer Drugs." *Journal of Economic Perspectives*, 29(1): 139-62.

^{viii} An Economic Evaluation of the War on Cancer Eric C. Sun, Anupam B. Jena, Darius N. Lakdawalla, Carolina M. Reyes, Tomas J. Philipson, and Dana P. Goldman NBER Working Paper No. 15574 December 2009. We arrived at the number of life-years gained using the approach in this paper and updating it. We multiplied the average increase in life years (2 million) by the number of years between 1995 and 2013. (18) 2x18=36 million We then multiplied the additional life-years by a conservative estimate (\$82,000) of what people think (in dollar amounts) they would gain by living another year. 36x82=2.952 trillion.

^{ix} [IMS Health Finds Global Cancer Drug Spending Crossed \\$100 Billion Threshold in 2014](#)

<http://bit.ly/1F4hEmT>

*Agency for Healthcare Research and Quality. Total Expenses and Percent Distribution for Selected Conditions by Type of Service: United States, 1996-2012. Medical Expenditure Panel Survey Household Component Data. Generated interactively.(June 09 2015)
<http://1.usa.gov/1ciYL2a>

^{xi} http://www.nytimes.com/2015/01/15/opinion/why-drugs-cost-so-much.html?_r=0

^{xii} http://www.nytimes.com/2015/01/15/opinion/why-drugs-cost-so-much.html?_r=0

^{xiii} <http://bit.ly/1F4mKQ0>

^{xiv} Indication-Specific Pricing for Cancer Drugs <http://bit.ly/1F4mXTk>

^{xv} **Patent Protection, Market Uncertainty, and R&D Investment**
[Review of Economics and Statistics](#) (Impact Factor: 2.66). 02/2008; 93(1).
DOI: 10.2139/ssrn.92562

^{xvi} Tatjana Nabokin: Global Investment Decisions and Patent Protection: Evidence from German Multinationals Munich Discussion Paper No. 2014-40 Department of Economics University of Munich Volkswirtschaftliche Fakultät Ludwig-Maximilians-Universität München Online at <http://epub.ub.uni-muenchen.de/21266/>

^{xvii} [Ann Pharmacother](#). 2001 Sep;35(9):990-6.**Newness of drugs and use of HMO services by asthma patients.**[Horn SD](#)¹, [Sharkey PD](#), [Kelly HW](#), [Uden DL](#).

^{xviii} **Benefit Design And Specialty Drug Use** (with Geoffrey F. Joyce, Grant Lawless, William H. Crown, and Vincent Willey), *Health Affairs* (2006)

^{xix} Seabury S, Goldman D, Kalsekar et al. "Formulary restrictions on atypical antipsychotics: impact on costs for patients with schizophrenia and bipolar disorder in Medicaid." *American Journal of Managed Care*, 20(2):e52-60, 2014

^{xx} Howard, David H., Peter B. Bach, Ernst R. Berndt, and Rena M. Conti. 2015. "Pricing in the Market for Anticancer Drugs." *Journal of Economic Perspectives*, 29(1): 139-62.

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xxiii Agency for Healthcare Research and Quality. Total Expenses and Percent Distribution for Selected Conditions by Type of Service: United States, 1996-2012. Medical Expenditure Panel Survey Household Component Data. Generated interactively.(June 09 2015)
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xxiv Agency for Healthcare Research and Quality. Total Expenses and Percent Distribution for Selected Conditions by Type of Service: United States, 1996-2012. Medical Expenditure Panel Survey Household Component Data. Generated interactively.(June 09 2015)

xxv [Using Disability Adjusted Life Years to Value the Treatment of Thirty Chronic Conditions in the U.S. from 1987-2010](#)Tina Highfill and Elizabeth Bernstein - BEA Working Paper (WP2014-9)

xxvi If this combination provided the same benefit to all other cancer types (it would eliminate other costly and less effective care. The average cost of caring for someone with cancer in their last year of life is \$104000. Average obtained with data from <http://costprojections.cancer.gov/annual.costs.html> found in "Projections of the Cost of Cancer Care in the United States: 2010–2020" by Mariotto et al. [J Natl Cancer Inst 2010; 103(2): [117-128](#)].

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xxix http://www.nytimes.com/2015/01/15/opinion/why-drugs-cost-so-much.html?_r=0

xxx <http://bit.ly/1F4mKQ0>

xxxi Indication-Specific Pricing for Cancer Drugs <http://bit.ly/1F4mXTk>

xxxii Hult, Kristopher J. "Incremental Innovation and Pharmaceutical Productivity_" December 31, 2014 http://home.uchicago.edu/~khult/jmp_draft.pdf
xxxiii Stevens W, Philipson T, Khan Z, MacEwan J, Linthicum M, Goldman D. "Cancer mortality reductions were greatest among countries where cancer care spending rose the most, 1995-2007." Health Affairs, 34(4):562-70, 2015.

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xxxv http://www.pharmatimes.com/Article/14-09-27/Oncologists_slam_regional_variations_in_drug_approvals_access.aspx

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Appendix A

THE VA SYSTEM IS THE WRONG MODEL FOR MEDICARE BENEFICIARIES

Critics of the Medicare prescription drug benefit argue that the benefit should be changed to work like the Veterans Affairs' (VA) prescription drug benefit. These critics suggest that Medicare beneficiaries would be better off if Medicare prescription drug coverage were administered by the federal government in the same way as the VA. However, many VA beneficiaries prefer to use other coverage for their medicines rather than rely on VA coverage.ⁱ In addition, recent data demonstrate that imposing a restrictive VA-type formulary on the Medicare program is unlikely to work for the nearly 40 million Medicare beneficiaries who get their prescription drug coverage from Part D.ⁱⁱ

Comparisons of Part D and VA Ignore That Many VA Beneficiaries Have and Rely On Other Sources of Drug Coverage.

A 2012 survey of VA enrollees shows that roughly 3.3 million veterans, or 42 percent of VA enrollees, have supplemented their VA drug coverage with Part D or private insuranceⁱⁱⁱ and the trends are increasing. This includes approximately 1.5 million veterans enrolled in a Medicare prescription drug plan, or 36 percent of Medicare-eligible veterans enrolled in the VA health care system. The same data also show that when VA beneficiaries have a choice between VA and other health coverage, a majority prefer not to be limited to the VA benefit. The share of VA enrollees who plan to use VA services primarily for prescriptions in the future has declined from 17 percent in 2005 to 8 percent in 2011.^{iv} Consistent with these results, VA enrollees report purchasing many prescriptions outside the VA system. In 2011, 34 percent of VA enrollees who reported any prescription use obtained them outside the VA system.^v

Formularies Offered by Private Part D Plans Offer More Robust Coverage than the VA Formulary.

- A recent study by the Lewin Group found that of the drugs most commonly used by the US population, 95 percent or more were covered by both of the two most popular Part D plans compared to only 78 percent covered by the VA formulary.^{vi}
- Research from RAND shows drugs excluded from the VA formulary are drugs regularly prescribed to seniors. The 84 drugs excluded from the VA National Formulary accounted for nearly one-quarter (24.7%) of all prescriptions dispensed to seniors among the 300 most common medications. By contrast, drugs excluded from even the most restrictive Part D plan accounted for only 12.6 percent of total prescriptions, and drugs excluded from the least restrictive Part D plan accounted for just 4.4 percent of total prescriptions.^{vii}

· _Given the greater breadth of coverage offered by Part D plans, it's not surprising that data collected by the VA shows the prescribing patterns of private doctors outside the VA, which include the doctors who prescribe drugs for Medicare beneficiaries, are very different from the prescribing patterns of VA employed physicians, whom VA enrollees are required to see.viii The VA acknowledges that "[e]nrollees may choose to have Medicare Part D coverage to obtain prescription drugs prescribed by non-VA physicians that are not on the VA formulary."ix

VA Program Shows VA Formulary Does Not Cover Many Drugs Prescribed by Community Physicians.

In 2003, a VA-sponsored program that allowed enrollees to temporarily fill prescriptions written by non-VA physicians through the VAX showed that up to 42 percent of prescriptions initially written by private physicians were for non-VA formulary medicines.

i "2011 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, March 2012, p. A-18.

ii Medicare Trustees, 2014 Report to Congress, p. 148, Table IV.B7.

iii "2011 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, March 2012, pp. A-1 and A-18.

iv "2011 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, March 2012, p. 57.

v "2011 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, March 2012, p 76; "2007 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, May 2008, p. 73.

vi The Lewin Group; 2013 Comparison of VA National Formulary and Formularies of the Highest Enrollment Plans in Medicare Part D and the Federal Employee Health Benefit Program, October 2013.

vii Joyce et al. "Medicare Part D After 2 Years," *Am J Manag Care*, August 2009.

viii Statement of Dr. Jonathan Perlin, Deputy Under Secretary for Health Department of Veterans Affairs; March 30, 2004 (Found at <http://www.va.gov/OCA/testimony/hvac/sh/040330JP.asp>)

ix "2010 Survey of Veteran Enrollees' Health and Reliance Upon VA," Department of Veterans Affairs, May 2011, p. 40.

x The transitional pharmacy benefit (TPB) was a temporary program to help veterans who were unable to get their initial primary care appointment with a VA doctor within a 30 day time period. Under the program, VA would fill prescriptions from private physicians until a VA physician examined the veteran and determined an appropriate course of treatment. The VA reported that 8,298 veterans had prescriptions filled through the program.

xi Statement of Dr. Jonathan Perlin, Deputy Under Secretary for Health Department of Veterans Affairs; March 30, 2004 (Found at <http://www.va.gov/OCA/testimony/hvac/sh/040330JP.asp>)



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