Evaluating industry's drug pricing claims

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Executive Summary

The Pharmaceutical Research Manufacturers of America (PhRMA) is a biopharmaceutical trade organization that represents 34 companies and their policy agendas in the U.S. and abroad.[1] It is widely known for its well-funded lobbying efforts, advertising campaigns and political donations that favor pharmaceutical manufacturers' positions in the U.S. market, including keeping drug prices high. Recent areas PhRMA has supported include policies that prevent Medicare from negotiating prices, block the establishment of formal health technology assessment groups to determine how much agencies should pay for drugs, and prohibit the U.S. from referencing prices abroad.[2, 3] In mid-September, PhRMA also launched a "seven figure ad campaign against the proposals moving through Congress to lower prescription drug prices".[4]

The U.S. tops the charts with its healthcare and prescription drug spending, yet ranks last among other Organization for Economic Cooperation and Development (OECD) countries in life expectancy, experiences the highest rate of avoidable death, and has the highest per capita spending on pharmaceuticals.[5, 6] Despite this data, PhRMA continues to tout the industry's leadership in research and innovation, productivity, and contributions to life expectancy, attributing such successes to the market-based system and the policy protections in the U.S. while pouring millions into lobbying ($29 million in 2019 to be exact, a record high).[7]

The Drug Pricing Lab developed three briefs to address PhRMA's main talking points.

- The first brief dispels the notion that innovation and R&D investment will fall precipitously if Congress takes action to rein in high drug prices.
- The second brief evaluates R&D productivity, and points to potential areas where R&D spending could be decreased without a negative effect on innovation.
- The third brief addresses the health outcomes that arguably should be the primary goal of this investment and innovation—and how the U.S. compares to other high-income countries.

While there is no doubt biopharmaceutical manufacturers have made important contributions to public health, it is imperative that policymakers have a comprehensive picture of the evidence surrounding industry claims to create policies that align drug prices with the clinical value they provide.

CLAIM 1: Lower drug costs will dramatically reduce R&D investment and innovation

What PhRMA says: PhRMA often emphasizes how much the biopharmaceutical industry invests in and spearheads research and development.[8, 9] The emphasis on solely industry's contribution to research and funding perpetuates an idea of self-sustenance; that the prices pharmaceutical manufacturers charge for medicines, particularly specialty drugs that can range anywhere in the hundreds to millions, generate revenue that is recycled back into the respective companies where an army of in-house scientists work diligently to discover the next miracle product. Put another way, the high prices charged incentivizes manufacturers to innovate, and a threat to the status quo is therefore a threat to innovation. PhRMA uses this argument to support policies (and policymakers) that give manufacturers the autonomy to set their own launch prices, because this is the only mechanism by which a competitive market can work.

What actually happens: There is no doubt the industry is a large funder of R&D, and these investments are indeed a critical element in bringing new medicines to market. Lower prices are likely to reduce sales, since the inelasticity of the market means that lower prices are not likely to lead to an offsetting increase in volume. That being said, there are plenty of revenues and profits to absorb moderate reductions and still fully fund R&D.

Moreover, biopharmaceutical companies are not the only entities funding drug research. A very significant portion of investment comes from public contributions for early-stage research, which companies rely on and build upon to develop and market commercial products. The financial rewards for industry are huge, largely due to the companies' ability to support late-stage development and commercialization of the products, boosted by the opacity of the drug pricing system.

Countering the industry's claim that negotiating lower drug prices will limit access, the truth is that the high prices currently charged for medicines (particularly expensive biologic and specialty medicines) are a major impediment to access, and one of the main drivers behind the need for drug pricing reform.

The following sections explore how R&D investment works and how industry stays on top.

What is R&D and who are the main investors?

R&D in drug development has several phases, starting with the pre-clinical phase, which often uses animal models to assess safety and risk and includes basic research, before going into phase I with human volunteers. Clinical trials generally take about seven to 10 years from pre-clinical to market, and cost anywhere from $0.8 billion to $2.3 billion of R&D per new drug.[10] PhRMA estimates that the pre-clinical phase typically accounts for 16% of R&D spending, Phase I 9%, and Phase II 10%, with the most expensive aspects of R&D during Phases III (29%) and IV (11%) on large-scale human subjects. The remaining 25% is spent getting a drug to approval or falls under miscellaneous expenses.[11]

These estimates include the costs of laboratory research, as well as resources spent on drugs that ultimately fail, are withdrawn, or do not receive FDA approval. Because most molecules never make it beyond the pre-clinical phase, investing in research and subsequently failure is difficult to quantify. It is precisely this inability to fully quantify "research" that PhRMA uses to justify charging premiums on existing drugs and argues that innovation is at risk if drug prices are cut.

Funding for R&D comes from both the public and private sectors, typically during different phases of development. A large portion of investment in basic research comes from the federal government, either directly or indirectly through grants, university research, subsidies, or other public awards. In 2019, approximately $31.4 billion or 80% of the National Institute of Health's (NIH) R&D budget was allocated to external research groups, making the NIH one of the largest government funders in the world.[12]

This investment in basic research is foundational to new drug approvals. It generates knowledge that may not be specific to any one marketable product but sets the groundwork for industry's privately funded research.[10] One study found that all 210 new drugs approved by the FDA from 2010-2016 was derived from published research funded by the NIH.[13] Government funding for foundation-level research is not uncommon. Other industries, such as those for semi-conductors and other technology, also receive public funding for research, where private companies then build on these findings to create and profit from new products.[14]

Private sector R&D investment typically occurs during the drug development phases. Venture capital investments in R&D, while a smaller share of total R&D funding than industry and government, are usually geared toward emerging companies with little revenue at the earliest stages of drug development. In 2020, biotech and pharma investments reached a record level of $28.5 billion.[15] At 17% of total venture capital funding, this level of investment highlights the attractive returns produced by the biopharma industry.

The largest funder in drug development is the pharmaceutical industry. The industry spent about 25% of their revenues on R&D in 2019, amounting to $83 billion.[10] This share is twice as much as what they invested 20 years ago, which coincides with the increasingly higher costs of running later stage trials. In recent decades, industry's investment dollars have shifted from in-house early-stage research towards later phases of drug development when a potentially marketable product exists and testing can begin.

How has the investment landscape changed?

Two increasing trends emerging from industry to secure portfolios and replenish pipelines are outsourcing licensing deals to other countries, particularly China, and acquiring and in-licensing promising early-stage companies and products to augment internal R&D pipelines.[16, 17] As of 2019, more than 70% of nearly 3,000 drugs in Phase III trials were being developed by small drug companies (revenues less than $500 million).[10] Conversely, only 23% and 11% of Pfizer and Johnson & Johnson's products, respectively, were developed in-house.[18] The cost of mergers and acquisitions reached a record high of $342 billion on deals in 2019, driven by a few big purchases, such as AbbVie's $63 billion purchase of Allergan and Amgen's $13.4 billion acquisition of the psoriasis drug Otezla.[19]

This market segmentation can be mutually beneficial as large companies are better equipped to streamline financial, administrative, and marketing functions, and small companies with less overhead can concentrate on research. Thus, almost half of R&D spending by big companies are for late phase and post-marketing trials to extend their products and indications.[11] However, not all deals are meant to spur innovation and pad pipelines; evidence also shows that acquisitions, particularly for early stage products, are frequently motivated by the desire to limit competition, especially when the acquirer's existing portfolio holds market power and competition is scarce.[20]

Beyond what might be considered more traditional R&D investment, court documents have exposed other ways in which industry prioritizes marketing under the guise or in favor of R&D. Marketing trials, also known as "seeding trials", may be categorized by companies as R&D, but they are purely marketing tactics, which muddies the waters and makes it more difficult to accurately quantify R&D. Two notable examples include PhRMA member companies Merck and Parke-Davis, whose marketing departments designed and implemented trials masquerading as safety research, when their primary objectives were to accustom physicians to prescribe their products.[21, 22]

Manufacturers' prioritizing marketing efforts, with budgets that appear to dwarf their own R&D budgets, have come under fire in recent years. The reason for all this funding? Because marketing campaigns work. Spending on direct-to-consumer (DTC) marketing, which began in the 1990s, has more than quadrupled from $1.3 billion in 1997 to $6.6 billion in 2020.[23, 24] DTC advertising has increasingly focused on costly biologics and cancer drugs to drive revenue, and some of the most aggressive spending targets drugs with little to no therapeutic gain.[23, 25, 26] In 2015, the American Medical Association called for a ban on DTC ads, citing concerns that they are driving up demand for expensive treatments despite the availability of cheaper, equally efficacious alternatives.[27] The Senate Finance Committee also took to questioning the marketing budgets of large pharmaceutical companies in a drug pricing hearing in 2019.[28] In 2020, AbbVie spent nearly half a billion dollars on DTC advertising for Humira alone, while the industry overall spent an estimated $6.6 billion.[23, 29]

PhRMA has since rebutted the claims that budgets are larger for marketing than research.[30] Regardless of the dollar amount, it is evident that the investment landscape has changed, and the result is a relationship so strong that some industry executives have voiced concern over previous NIH budget cuts, noting that "…adequate funding for the NIH is critical for the health of the nation. The relationship between industry and government funded research is symbiotic."[31]

What is the return on investment for approved drugs?

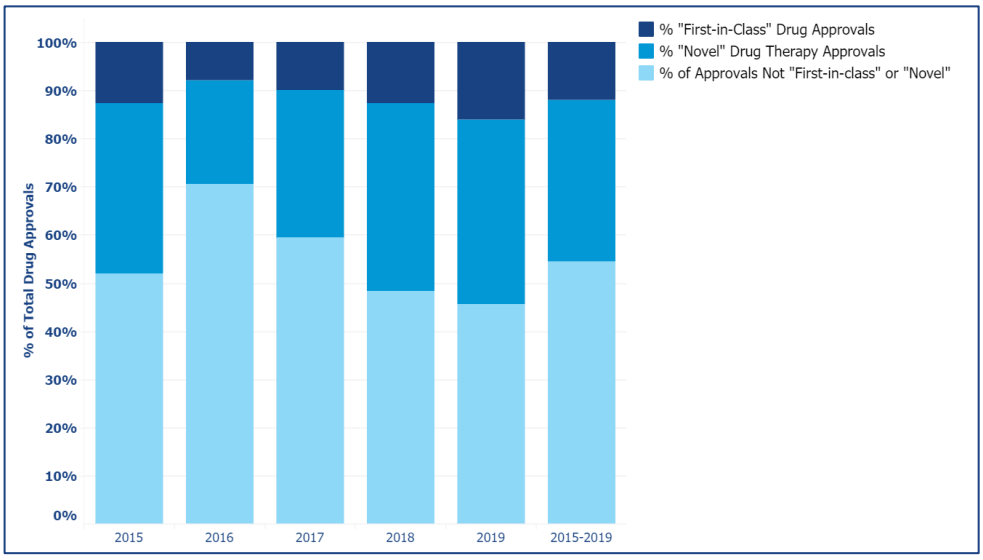
While the funding relationship is interdependent (i.e., government needs industry to develop products, industry needs government investments into researching novel mechanisms and pathways that are otherwise too risky for one company to take on alone), the financial rewards skew heavily in favor of industry. A study from 2000 to 2018 showed the median gross profit for the top 35 pharmaceutical companies on the S&P 500 was 76.5% compared to 37.4% for other large companies, median EBITDA 29.4% compared to 19.0%, and median net income of 13.8% compared to 7.7%.[32]

These outsize profit margins are driven by the manufacturer's ability to price drugs autonomously during the monopoly pricing period awarded by the FDA to newly approved drugs. During this time, the manufacturer's objective is to capture as much of the market share as possible before competitors enter. However, there are plenty of legal tactics manufacturers use to prevent competition from happening (for more on abuses of government programs and regulations, see DPL Challenge) and instances where cheaper alternatives do not drive down prices.[33, 34]

A review of company financial filings shows that revenues generated from excess pricing in the U.S. are enough to fully cover global R&D spending as well as large profit margins. Some drugs become such behemoths that a single product carries the entire corporation, even well beyond its exclusivity and patent expiration periods. A 2015 analysis showed the top-selling products of three companies (AbbVie with Humira, Biogen with Tecfidera, and Teva with Copaxone) would have covered all or nearly all of their research spending through premium pricing.[35] Manufacturers throw their weight behind these products with aforementioned marketing strategies, gifts and payments to prescribers, and tie cash bonuses to the sales of a singular product to drive volume.[36]

For the early-stage investors (i.e., the government and in turn the taxpayers), minimal direct financial returns from drug sales are trickled back to them, as any royalty streams tend to be modest relative to product sales. And with the increasing drug prices at launch, a portion of which insured patients pay as coinsurance, coupled with the fact that U.S. drug prices are higher than other wealthy nations, has led to the notion that Americans "pay twice" for a drug. For reference, the median price of newly approved cancer drugs increased 7-fold over 20 years, from $1,932 between 1995-1999 to $14,950 between 2015-2019, and the average coinsurance ranged anywhere from 18% to 28% of the list price in 2020, leaving patients with out-of-pocket costs anywhere from a few hundred to thousands of dollars.[37, 38] In some cases, patients are paying for a drug that has only incremental value in survivorship or quality of life, an issue of increasing concern as more drugs are approved through an expedited pathway (more on this in Brief 2).

Exhibit 1: Novel Drug Approvals as a Percent of Total FDA Approvals, 2015-2019



Source: DPL Challenge[33]

The unsustainable drug pricing market has made prescriptions unaffordable for many Americans and is forcing policymakers to find solutions. Yet, any attempt to thwart the status quo continues to be met with industry-induced scare tactics, like this video sponsored by PhRMA that improperly implies Medicare negotiation "cuts off access" to all life-saving medicines (it is important to note that all wealthy nations negotiate drug prices and still have access to effective drugs in each therapeutic class).[39] Industry also vehemently attacks any attempt to tie clinical value with drug price, ferociously pitting themselves and patient advocacy groups against organizations like the Institute for Clinical and Economic Review (ICER), an independent nonprofit group that conducts cost-effectiveness analyses of medical technologies and is a proponent of using quality-adjusted life years to measure health outcomes.[40]

There is plenty of evidence to refute the industry's claim that drug price reform will undermine innovation and access. While it is probably true that overhauling existing market dynamics may "force established companies to make difficult choices about which promising medicines to pursue or abandon," this is what they should do, and it wouldn't likely cut into the most promising and innovating aspects.[41] Between 2015-2019, only 12% of FDA approved drugs were classified by the agency as "first-in-class" and only 34% were considered "novel". Presumably the expenditures on the remaining 55% of FDA approvals present opportunities to reprioritize this spending (Exhibit 1).[33] Moreover, the out-of-pocket burden remains the greatest impediment to access, so it is disingenuous to suggest that lowering list prices (to which co-insurance is linked) would have the effect of further limiting access to innovating medicines.

CLAIM 2: R&D productivity justifies the status quo

What PhRMA says: The increasing number of drugs approved by the FDA each year and the thousands in development are often lauded by PhRMA as markers of excellence—the fruits of the industry's strong investments in R&D.[42, 43] Emphasizing the sheer number of drugs available, and soon-to-be available, implies that each one fills an unmet medical need, with new ones better than the old, and the ability for industry to churn out this level of production is only made possible in the U.S. As such, a threat to this policy environment is a threat to global innovation.

What actually happens: The U.S. biopharmaceutical industry has brought an increasing number of new drugs to market in recent years, and several of these have represented important breakthroughs for patients. However, the implication that high drug prices and exorbitant spending are directed towards new treatments is misleading. Many of the approved molecules are not necessarily more innovative than ones that already exist, and manufacturers continue to charge high drug prices for uncertain clinical improvement.

Moreover, in the past decade, there has been a notable trend toward developing products for so-called "orphan diseases", which have patient populations of less than 200,000 in the U.S. and come with tax credits. In 2020, 58% of all novel drugs approved were for the treatment of rare or orphan diseases.[44] These drugs generally undergo a less rigorous approval process, leading to earlier market access at high prices. The confluence of these trends and incentives suggest industry is exploiting this policy and exacerbating the drug pricing crisis many Americans are facing today (Exhibit 2).

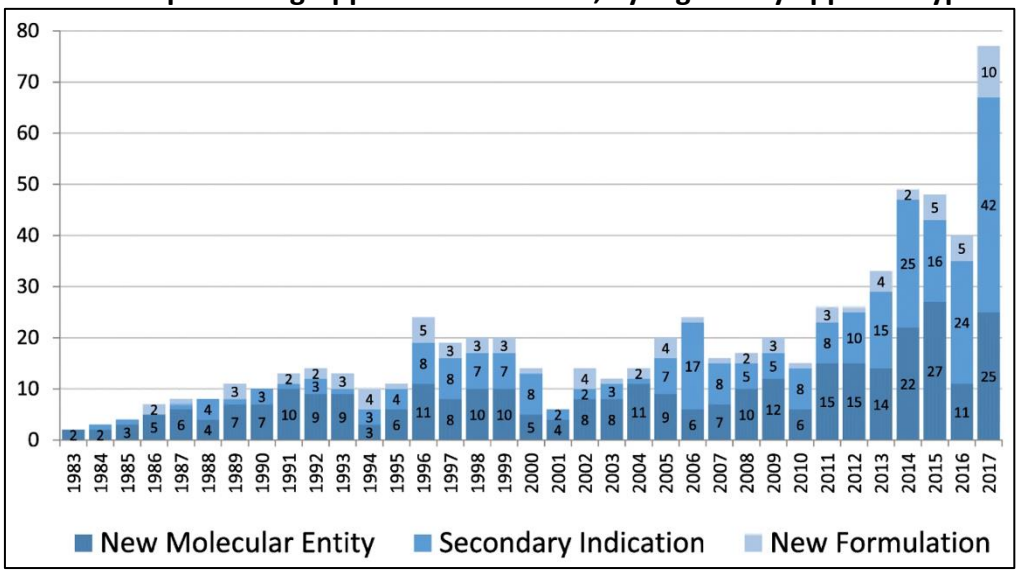
A sizeable percentage of the industry's R&D spending is not directly funding the clinical trials underpinning the development of new medicines. According to data published by PhRMA, between 11%-12% of annual R&D is on trials for already marketed products—more than what is invested in either Phase I or Phase II trials. Additionally, 21% of the industry's R&S spend is "uncategorized", suggesting that roughly a third of the R&D budget is not directly spent on products in development.

The following sections explore the quality of drugs approved.

How to measure productivity and what does it mean for patients?

During the drug approval process, the FDA classifies applications under one of 10 codes based on certain characteristics of the product and its relationship to drugs already approved in the U.S. market.[45] While the codes themselves are not indicative of innovation or value, they are a useful proxy to assess the types of drugs approved in a given year. One such classification is the new molecular entity (NME), which requires that the drug under review contain active moieties not previously approved and are frequently drugs that come to market as important new therapies for patients.[46] There are two ways to measure innovation through NMEs: one by the number of NMEs approved each year and the other through the sub-classifications of these NMEs (e.g., first-in-class, advance-in-class, and addition-to-class).

Exhibit 2. Orphan Drug Approvals 1983-2017, by regulatory approval type



Source: Miller 2018[47]

Approvals of novel drugs have increased over time, from an average of 25 NME approvals each year from 2000 to 2010 to an average of 41 per year the following decade.[46] This increase, however, coincides with a reliance on expedited pathways for marketing approval, which requires less clinical evidence for promising new drugs that target unmet medical needs and, in some cases, ask that manufacturers provide post-marketing trial results. The FDA designed four approaches to expedite the application process (fast track, priority review, accelerated approval, and breakthrough therapy), with a separate designation for drugs that treat rare diseases, derived from the Orphan Drug Act.[48, 49] These various designations and pathways are not mutually exclusive, with many drugs receiving multiple designations. A notable example is Gleevec (imatinib), first approved for chronic myelogenous leukemia and approved in less than 3 months under multiple designations.[50]

However noble the intent, expedited pathways also have their share of controversy given the nature of shortcutting rigorous review processes. Many have suggested that the increased reliance on surrogate endpoints and the growing use of single-arm studies for approval under the accelerated approval pathway, coupled with the high prices of drugs approved under this pathway, are causes for concern on access, cost, and innovation.[50]

The increase in approvals using expedited pathways are also being driven by drugs that are not first-in-class. Between 1987 and 2014 there was a 2.4% annual increase in the number of new drugs approved through at least one of the four expedited approval or review pathways.[51] These findings suggest productivity trends for innovative drugs are more muted than what industry claims.

Many concerns surrounding these pathways have been widely discussed in the press surrounding Biogen's new drug Aduhelm (aducanumab) used to treat Alzheimer's disease. In June 2021, the FDA controversially granted accelerated approval to Aduhelm based on inconclusive clinical trial data and a negative recommendation by the FDA advisory committee.[52] With access to the market and no supporting evidence of effectiveness, Biogen will be able to charge Medicare approximately $56,000 per patient per year, more than twice as high as the Institute for Clinical and Economic Review's (ICER) value-based price, which assumed the most optimistic estimate for Aduhelm's efficacy.[53]

Who wins when data are shortchanged? And who loses?

Well-designed clinical trials are essential to high-quality research and discovery of novel treatments, with core components including randomization, study control(s), robust sample size, and reproducibility. The FDA relies on trial results for drug approvals; however, regulation has relaxed overtime, shifting from requiring two "adequate and well-controlled trials" to one trial with confirmatory evidence in situations where a second trial would be "practically or ethically impossible".[54]

Since the guidance allowing single trial evidence for approval was published in 1998, the number of NME approvals based on a single trial grew from 37% in the 2005-2012 period to 42% by 2018, of which 28% were solely based on the results of phase I or II trial.[55, 56] Approving drugs based on a single trial raises questions about the reproducibility of the reported results. Out of the 35 NME approvals based on a single trial between 2000-2016, only 66% demonstrated consistent results across subgroups and 43% demonstrated consistent results across clinical endpoints.[57]

Even when drugs are found to be ineffective in confirmatory trials following approval, the FDA has been reluctant to revoke approval status, allowing drugs to stay on the market in perpetuity.[58] Proamatine (midodrine hydrochloride; manufactured by Takeda Pharmaceuticals) is one example where the FDA decided to withdraw its marketing approval after inadequate post-marketing data in 2010. Public opposition, in part stirred up by individuals and advocacy groups with close financial ties to large pharmaceutical companies, caused the FDA to reverse their decision. Proamatine remains on the market without full approval 24 years after its initial accelerated approval.[50]

Because clinical trials are expensive to implement, less rigorous data requirements save manufacturers money. Pivotal trials are estimated to cost an average of $19 million dollars, $35.1 million for those with a control and $13.5 million for those without.[59] Moreover, costly blockbuster drugs are more valuable for a company if they can be tested for expansion into new indications or drug combinations.

Although more lucrative for a company, running a large portfolio of trials can lead to underpowered trials with exaggerated treatment benefits, and has the potential to strain future clinical trial accrual as studies pull from a limited pool of patients.[60, 61] Clinical trial participants are a scarce resource and one of the largest drivers of clinical trial cost, which further illustrates the importance of allocating participants to trials adequately designed to test safety.[62, 63]

Companies have also turned overseas to recruit and conduct trials to address these issues of high cost and patient supply. However, most drugs that received FDA approval using trial participants in low- and middle-income countries were not always made available in those countries even within 5-years after U.S. approval.[64] This not only raises equity concerns but also coincides with manufacturers' market access strategies that often targets the U.S. first and delays launch in countries that use external reference pricing or have less marketing potential.[65]

Industry claims high drug costs pay for R&D and innovation. Yet, data shows that manufacturers make decisions that maximize profits even at the expense of public health, and the results can be damaging. For

cases like Aduhelm, Proamatine, and many others that have questionable benefit for patients, regulatory bodies and manufacturers have a responsibility to uphold scientific standards and minimize preventable casualties, not the other way around.

CLAIM 3: R&D innovation extends lives, improves human productivity, and reduces healthcare costs

What PhRMA says: To further justify high prices, PhRMA often emphasizes their contributions to life expectancy, human productivity, and societal gains.[66] This argument plays on the talking points from briefs 1 and 2. Prescription drugs treat ailments that would otherwise inhibit productivity, which directly leads to healthier and longer lives. In addition to the thousands of drugs on the market and in development giving hope to a promising future, most life expectancy gains in the U.S. and abroad, a measurable impact, are largely attributed to biopharmaceutical treatments.[67]

What actually happens: Biopharmaceutical innovation has advanced in recent decades and is in many respects responsible for drastically improving quality of life and even eradicating some of the world's most debilitating diseases. Such drug discoveries, however, are only part of the equation. Improved quality of life and life extension is largely attributed to public health interventions and screening. Further, high drug prices continue to financially strain patients, especially those with comorbidities, while inflating insurance premiums for everyone. This leads to unequal access to treatment, particularly for those in a lower socioeconomic bracket, of which many are people of color.

Among wealthy nations, the U.S. fares last on nearly all measures of life expectancy despite being one of the largest government funders in the world for R&D, having access to the newest therapies before other countries, and covering more approved drugs on the market at any given time. When innovative drugs are inaccessible, the policies sustaining high drug prices need to be evaluated.

The following sections explore the relationships between drug prices, access, and life expectancy.

What factors impact life expectancy and how does the U.S. fare against other countries?

Life expectancy is affected by general living standards, such as crime, hygiene, education, average income, and other societal factors. In the U.S., the average life expectancy increased from 75.2 years in the 1990s to 78.8 years in 2014, when the rate of increase stalled, before declining to 78.5 years in 2016. In other high-income countries, life expectancy has been consistently above 80 years over the same time period, hovering from 82 to 84 years.[68] High incidences of car accidents, gun violence, health care inequity, and opioid use are unique to the U.S. and some of the largest contributors to the lower averages.

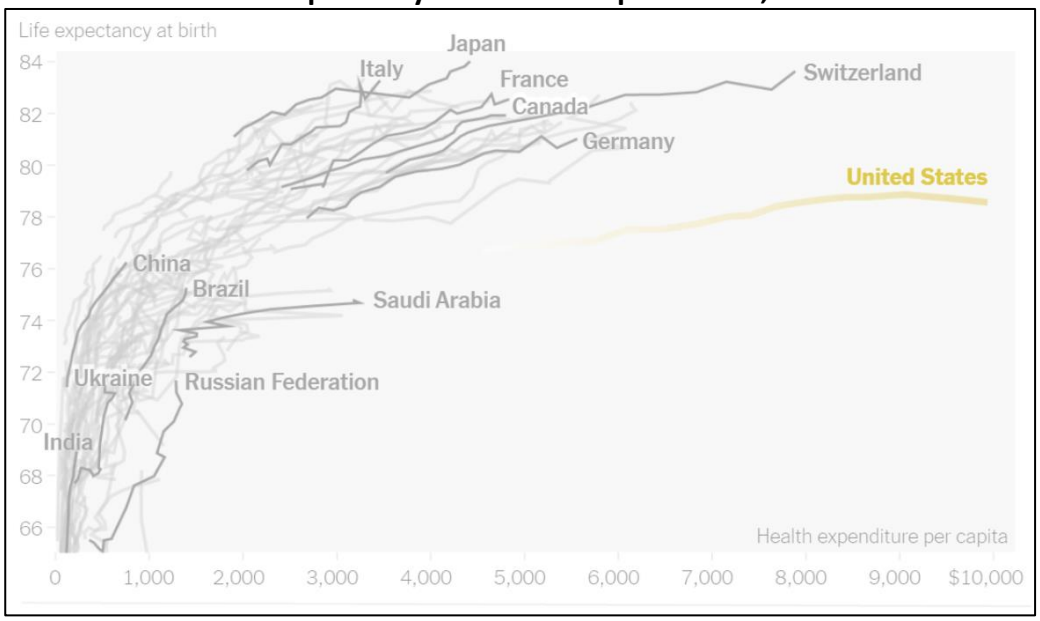
Public health interventions also impact life expectancy, arguably more so than pharmaceutical interventions. In one analysis, 44% of the improved 3.3-year increase in life expectancy from the 1990s through 2015 was attributed to these interventions compared to only 35% from pharmaceuticals, a far cry from the 75% PhRMA claims.[67, 69] Other studies have found that for every 10% increase in public health spending, mortality rates dropped between 1% to 7%.[70] And yet, spending on preventative measures accounted for only 2.9% of U.S. health spending in 2018 while pharmaceutical spending was 12.4%.[6, 71]

The impact of public health interventions is much more apparent in some disease areas. U.S. cancer mortality rates have declined from 1991 to 2017, and has resulted in an overall drop in mortality of 29%, or approximately 2.9 million fewer cancer deaths during this period.[72] These declines can largely be attributed to early detection through screening and smoking cessation, aided by public health messaging, behavioral changes, and new oncology drugs.[72, 73]

Variability within the life expectancy measure: Why is the U.S. unique?

The U.S. is typically the first country to approve and have access to new medicines, and to spend the most on health care and prescription drugs; yet, it consistently ranks last among wealthy nations in life expectancy (Exhibit 3).[65] It is the only high-income country that does not provide a form of universal basic health care to all its citizens. Instead, insurance schemes are privatized, with many plans requiring high patient cost-sharing (on average 20% to 30% of the drug's list price) for lower premiums. As a result, access to care remains a pipe dream for many Americans, and an even more cumbersome feat for those with co-morbidities, chronic diseases, or are without insurance. In the country that has one of the largest government funders in R&D in the world propped by taxpayer contributions, targeted policies that foster and protect pharmaceutical innovation, and plenty of approved prescription drugs on the market, how do millions of Americans struggle to access prescription drugs?

Exhibit 3. Global life expectancy and Health Expenditures, 2000-2017



Source: World Bank [74]

Industry is quick to blame payers and pharmacy benefit managers (PBMs). High copayment terms and formulary restrictions, designed around economic incentives rather than clinical value, can prevent patients from accessing the treatments they need. These decisions are justified by payers and PBMs to steer patients away from high cost drugs when lower alternatives are available. However, this relationship between payers and PBMs and manufacturers evolved in tandem. Manufacturers, by charging a high price at launch, sets the benchmark throughout the rest of the supply chain. Payers and PBMs then negotiate a rebate from manufacturers for more favorable formulary placement, and each party profits when the price of the drug

is high, except patients, who are the only stakeholders in the drug supply chain that pay based on the drug's list price instead of its net price after rebates and discounts.

With no regulatory requirement or formal governing group assessing health technologies, no standard methodology exists to compare the effectiveness of new drugs against competitors on the market, which ultimately allows for the approval of drugs with different levels of effect, including those with marginal health gains. In one study of drugs approved between 2002 and 2014 for patients with solid tumors, the median gain in overall survival was 2.1 months and only 42% of those approvals were found to result in "clinically meaningful improvements" in outcomes according to goals set by the American Society of Clinical Oncology.[75] The industry's true incentive is to maintain high prices even for products with questionable clinical efficacy, and the U.S. is the industry's playground given its unique inability to control prices. It is no wonder PhRMA vehemently opposes any policy that aims to disrupt this status quo.

For patients, this typically means access to drugs are limited for those who are financially secure and reasonably close to a prescriber and pharmacy. Since 2001, the poorest 5% of Americans experienced close to zero gains in survival, while middle- and high-income Americans have gained over 2 years in additional life expectancy.[76] Put another way, differences in life expectancy between the wealthiest 1% and poorest 1% were 14.6 years for men and 10.1 years for women.[76]

The legacy and persistence of institutionalized racism in the U.S. exacerbates the issues of access and further divides life expectancy metrics. White patients are consistently more likely to have better health outcomes and longer life expectancy than Black patients due to more favorable affordability, accessibility, prescribing biases, and even enrollment opportunities in clinical trials. Inequalities percolate through to other social determinants that are not always immediately observable or quantifiable but are ultimately reflected in the aggregate. Racism and financial hardships limit access to education, food, housing, and transportation, which can increase the risk of chronic stress, disease, and disability, transcending across generations and perpetuating poor health outcomes.[77, 78]

This is the pharmaceutical market in the U.S., and it does not work for many patients, evidenced by lower life expectancy rates compared to other wealthy countries. The purported objective of pharmaceutical innovation is to innovate for patients in need. However, the health care system in the U.S. has evolved to favor industry profiteering over public health, and the results are obvious from all angles.

Conclusion

PhRMA consistently pushes the narrative that manufacturers are the main drivers of R&D investment, productivity, and societal gains in life expectancy, and that legislative changes aimed at regulating their ability to autonomously price drugs or increase transparency on their business strategies would significantly hinder innovation and stain the reputation of American exceptionalism in medical research.

In reality, this narrative is fraught with inconsistencies, factual errors and half-truths, and other misleading statements. The messaging also ignores the multi-sector and global partnerships that have invested, studied, and revolutionized modern medicine. The current infrastructure of pharmaceutical R&D involves numerous participants and funding sources, a significant proportion of which comes from taxpayer money.

Moreover, a closer look at the investments made by these companies show that they are clearly directing the lion's share of their spending on late-stage development and profit-seeking around existing products—contrary to what is generally thought of as "innovation". Companies are mitigating substantial risks associated with clinical research by in-licensing products discovered by small biotech companies and foreign countries, and increasingly advocating for and taking advantage of less rigorous scientific standards to obtain FDA approval quickly and more cheaply. This rise in expedited approvals combined with the unrestricted ability for companies to set prices (a lever unique to the U.S.) has resulted in more and more approvals for unsustainably priced drugs with marginal clinical benefit.

Pharmaceutical companies are private-sector corporations and act as such to maximize revenue and lower business costs, oftentimes at the expense of public health and budgets. Rather than uncritically accepting PhRMA's talking points, policymakers need to understand, objectively, how the U.S. system "works", which stakeholders are winning, and most importantly, how patients are losing. A new approach is urgently needed—with policies that incentivize true innovation by aligning drug prices with the actual value they provide to patients and society.

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