

# CSRxP: Proposals for Change

The current market for pricing drugs in the United States is broken, and it's unsustainable for American businesses, families, and our economy. Prescription drug prices are needlessly high and continue to increase at unsustainable rates – even as far too many Americans currently face the severe health and economic consequences from the ongoing COVID-19 pandemic. Today, prescription drugs make up the largest expense of every health care dollar, comprising 21.5 cents of every health care dollar spent, and spending on prescription drugs has grown faster than most other areas of health care in recent years.<sup>1 2</sup>

Excessively high drug prices unfairly threaten the financial security, health and wellbeing of U.S. patients and their families on a daily basis, while also straining Federal and state health budgets and the taxpayers who fund them. That's why the Campaign for Sustainable Rx Pricing (CSRxP), a nonpartisan coalition of organizations, is fostering a national dialogue focused on the issue of drug pricing that strikes a balance between innovation and affordability. CSRxP members represent hospitals, physicians, nurses, consumers, health plans, pharmacists, and employers who have joined together to put forward bipartisan, market-based solutions that promote **affordability, competition, transparency, and value** to restore a functioning prescription drug market for all American consumers and taxpayers.

**Affordability:** Too often patients experience the unfortunate and unfair choice of purchasing the medications they need to be well and paying for other necessities. Patients should never be presented with such a choice – especially today with so many people across the U.S. experiencing significant health and economic hardship due to the COVID-19 pandemic. Indeed, one in four Americans already could not afford their medications *before* the COVID-19 public health crisis began – and many of these patients reporting challenges with affording their medications were in fair or poor health and had low incomes.<sup>3</sup> Taxpayers also are confronting the unaffordable and unsustainable growth in prescription drug spending; drug costs in federal health programs including Medicare and Medicaid have increased substantially and are forcing a greater portion of the national budget each year to be dedicated to health care. Measures must be taken to improve prescription drug affordability for patients and taxpayers alike, especially while the Nation continues to battle the COVID-19 pandemic and all people across the U.S. need access to acceptably-priced and affordable virus countermeasures.

- **Ensure Universal Access to Affordable COVID-19 Vaccines and Treatments with Full Transparency on Government Investment in Virus Countermeasures:** The COVID-19 pandemic has exerted severe health and economic strain across the U.S. Hundreds of thousands of Americans have died from the virus and millions have lost their jobs. To recover and come back stronger, it is critical that all people across the U.S. have affordable access to vaccines and treatments that combat the virus. Policymakers must ensure that the COVID-19 vaccine is as affordable as the annual flu shot and reflects the substantial investments made by taxpayers, and that distribution policies guarantee all Americans equal access to the vaccine. Antibody

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<sup>1</sup> AHIP. "[Your Health Care Dollar: New AHIP Analysis Shows Where It Goes.](#)" November 12, 2020.

<sup>2</sup> Pew Charitable Trusts. "[A Look at Drug Spending in the U.S.: Estimates and Projections from Various Stakeholders.](#)" February 27, 2018.

<sup>3</sup> Kaiser Family Foundation. "[Poll: Nearly 1 in 4 Americans Taking Prescription Drugs Say It's Difficult to Afford Their Medicines, including Larger Shares Among Those with Health Issues, with Low Incomes and Nearing Medicare Age.](#)" March 1, 2019.

treatments and other therapies that treat COVID-19 patients should be reasonably and affordably priced such that cost does not serve as a barrier to treatment for patients, regardless of whether they are enrolled in federal health programs like Medicare and Medicaid, have commercial insurance, or no insurance at all.

Moreover, recognizing the urgent need to develop and make these virus countermeasures available as quickly as possible, the Congress has authorized nearly \$10 billion to date for the development of COVID-19 vaccines and treatments.<sup>4</sup> From the funding, HHS has provided hundreds of millions of dollars upfront to certain biopharma companies for COVID-19-related research, treatment development, manufacturing, and product distribution. In exchange for furnishing this extensive funding, the public should have a fully transparent and clear understanding of the entirety of the Federal government’s investments in COVID-19 countermeasures, as this information is critical for assessing whether these vaccines and treatments are reasonably and affordably priced. As the Nation continues to confront the pandemic, the federal government alone should determine when the COVID-19 Public Health Emergency (PHE) has concluded and the associated PHE countermeasure pricing and access policies no longer apply; any entities other than the federal government attempting to determine that the PHE has concluded simply do not have access to the complete and comprehensive data about the virus necessary to make such a determination.

Additionally, since an overwhelming majority of people across the Nation will need to receive the coronavirus vaccine because of the public health and economic impacts, the government has a unique role to play in ensuring the price of the vaccine remains affordable to the health system. The Administration should require transparency for pricing of the vaccine, including any price increases after the PHE expires, so that policymakers can understand how vaccine costs will impact government expenditures and those of private health plans. Further, policymakers should require that vaccine manufacturers disclose the price of their vaccine to other European governments.

- **Medicare Drug Price Increases Should Not Exceed the Annual Rate of Inflation:** Medicare spending on prescription drugs has far outpaced spending on other parts of the program, growing annually by double digit percentages in recent years, in large part due increased utilization of high-priced specialty biologic therapies.<sup>5 6</sup> Price increases in particular have been primary drivers of overall prescription drug spending growth in Medicare. In Medicare Part B, for example, drug expenditures on separately payable drugs grew by an average annual rate of 10.7% between 2009 and 2017 and nearly two-thirds (6.9 percentage points) of that spending growth was due to an increase in price.<sup>7</sup> Similarly, in Medicare Part D, 20 of the top 25 drugs by

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<sup>4</sup> HHS. “[Fact Sheet: Explaining Operation Warp Speed](#).” Accessed November 10, 2020.

<sup>5</sup> Lieberman, S and Ginsburg, P. “[CMS’s International Pricing Model for Medicare Part B Drugs: Implementation Issues](#).” *Health Affairs Blog*. July 9, 2019.

<sup>6</sup> AARP. “[Rx Price Watch Report: Trends in Retail Prices of Specialty Prescription Drugs Widely Used by Older Americans: 2017 Year-End Update](#).” June 2019.

<sup>7</sup> Medicare Payment Advisory Commission June 2019 Report to the Congress: Medicare and the Health Care Delivery System. “[Chapter 3: Medicare payment strategies to improve price competition and value for Part B drugs](#),” pages 59 – 60.

total spending in 2017 had price increases ranging from 3 to more than 9 times the rate of inflation.<sup>8</sup> And some Part D therapies had price increases drastically higher than the rate of inflation – 15.7% for the pain medication Lyrica, 15.3% for the cancer treatment Revlimid, and 13.2% for the auto-immune therapy Humira Pen.<sup>9</sup>

The oftentimes enormous price increases implemented by drug makers are clearly unsustainable for both Medicare beneficiaries and taxpayers. Beneficiaries in many cases simply cannot afford the high out-of-pocket cost-sharing they face in Medicare Part B (20%) and Part D (as much as 33% before they reach the catastrophic phase of coverage) associated with expensive specialty medications. Taxpayers cannot be expected to continue bolstering the excessive profits of big pharma by paying for these unjustifiably high-priced therapies and inexplicable price increases. To put Medicare on a more sustainable fiscal path and better ensure beneficiaries can affordably access the medications they need, price increases for drugs covered by Medicare Part B and Part D should not exceed the annual rate of inflation.

- **Make Medicare Part D More Affordable:** The Medicare Part D prescription drug benefit has successfully expanded access to prescription drug coverage to America's seniors and disabled citizens who, in many cases, previously had none. In recent years, a growing number of high-cost specialty medications have been introduced into the market and subsequently have been covered under Part D. Drug makers have established high launch prices for these specialty therapies and imposed significant and frequent price increases after they enter the market. These expensive therapies have increased out-of-pocket spending substantially for far too many Part D enrollees who do not have a hard out-of-pocket limit. Specialty drugs also have significantly driven up the expense of the Part D program for the federal government, with growth in spending on specialty therapies far exceeding expenditure growth in non-specialty medications.<sup>10</sup>

To make drug coverage more affordable for Medicare beneficiaries, the U.S. Department of Health and Human Services (HHS) Office of the Inspector General (OIG) should immediately rescind the rule that limits Part D plans and pharmacy benefit managers (PBMs) from negotiating lower drug costs on behalf of Part D enrollees and needlessly raises Part D premiums by about 25% and imposes nearly \$200 billion in costs on taxpayers.<sup>11</sup> An annual cap on Part D out-of-pocket spending also should be implemented. Additionally, big pharma should be held much more accountable for the enormous cost growth it has contributed to the Part D program. Drug makers should assume liability for at least 50 percent of total costs in the catastrophic phase of the benefit and liability for a portion of costs in the initial phase of Part D coverage. Health plans should have more flexibility to manage high-cost Part D drugs, including drugs in the “protected classes,” while at the same time ensuring that meaningful, appropriate

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<sup>8</sup> Cubanski, J and Neuman, T. “[Assessing Drug Price Increases in Medicare Part D and the Implications of Inflation Limits.](#)” *Kaiser Family Foundation*. October 18, 2019.

<sup>9</sup> *Ibid.*

<sup>10</sup> Congressional Budget Office. “[Prices for and Spending on Specialty Drugs in Medicare Part D and Medicaid: An In-Depth Analysis.](#)” March 2019.

<sup>11</sup> [85 FR 76726](#)

beneficiary protections are in place. Part D regulations also should foster and promote beneficiary access to more affordably-priced generic and biosimilar therapies.

**Transparency:** Drug manufacturers regularly justify their pricing decisions by citing industry-funded research that claims that it costs \$2.6 billion to bring a new drug to market.<sup>12</sup> There is no way to independently verify this figure. Additionally, there is no way of determining how much pharmaceutical companies actually invest in research and development (R&D) activities as compared to marketing and advertising activities, for example. Allowing public access to this missing information could play a critical role in assessing whether a drug's price is, in fact, reasonable. There is also a dearth of information regarding other factors that pharmaceutical companies consider in setting launch prices or in deciding to raise prices for drugs that are already on the market. Furthermore, other stakeholders such as the government, employers, and insurers must price and budget many months in advance, leaving the entire system vulnerable to uncertainty associated not only with price changes for existing drugs, but also for prices and labels of new drugs that are not known until launch. Given the growing and significant impact pharmaceuticals have on overall health care spending, increased transparency is critical and cannot be dismissed with unfounded claims that any steps toward transparency will erode pharmaceutical investments in R&D.

- **Apply Price Transparency Parity:** As part of the approval process for new drugs, manufacturers should be required to disclose information regarding the maximum unit price they intend to charge for the product, the cost of a course of treatment, labeling information pending FDA approval indicating the drug's target population along with other important clinical details, and a projection of federal spending on the product. Subsequent to approval, manufacturers should be required to report, on an annual basis, any increase in the list price of that drug over a threshold as along with how many times a year the price of a drug has been increased. Information would be reported to HHS by drug manufacturers to enhance drug pricing transparency, but with protections in place to exclude sensitive, proprietary information. Policies such as these are why CSRxP specifically has endorsed the Prescription Drug Sunshine, Transparency, Accountability and Reporting (STAR) Act and the Fair Accountability and Innovative Research (FAIR) Drug Pricing Act in the 116<sup>th</sup> Congress – both important pieces of legislation that will require manufacturers to publicly disclose pricing information and justify price increases for their high-priced drugs. Indeed, CSRxP appreciates that the Consolidated Appropriations Act of 2021 promotes drug pricing transparency by mandating health plans to report annual drug cost increases and plan spending on prescription drugs, but believes that such reporting requirements will be burdensome for health plans and drug manufacturers are in the best and most appropriate position to report drug pricing information since they alone set list prices and implement list price increases.

Transparency requirements applied in healthcare contexts are notably not without precedent. Currently, many entities in the healthcare sector report pricing data to government entities. Health plan issuers are required to provide premium information to state insurance commissioners many months in advance of those premiums applied to plans in the market. In

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<sup>12</sup> DiMasi JA, Grabowski, HG, Hansen RA. [Innovation in the pharmaceutical industry: new estimates of R&D costs](#). *Journal of Health Economics* 2016; 47:20-33.

addition, issuers with rate increases above 10% are required to submit a justification to the government for review and must make summary information accessible to the public in an understandable format. Additional examples involving transparency requirements in our health care system address providers such as hospitals, skilled nursing facilities, and others that must submit cost data to HHS annually. This includes information on facility characteristics, utilization data, costs, charges, and certain financial data. It is time these transparency policies applied in other areas of the health care sector be extended to the pharmaceutical industry as well.

- **Guarantee a Better Return on Taxpayer Investments:** While high prices are often justified based on the costs associated with R&D, there is virtually no public data showing a link between prices and development costs. For example, the R&D for sofosbuvir – the active ingredient in the blockbuster medications used to treat Hepatitis C including Harvoni, Sovaldi, Epclusa and Vosevi – was largely conducted by a small biotech company (later acquired by a larger drug manufacturer) that received the majority of its funding from the National Institutes of Health (NIH). Gilead purchased this biotech and recouped its acquisition costs in one year of drug sales: the company launched Sovaldi and later Harvoni at \$84,000 and \$94,500, respectively, for a full course of treatment and, in the first year Sovaldi was on the market in 2014, Gilead astoundingly generated \$10.3 billion in sales alone from the product.<sup>13</sup>

Research indicates that funding from the National Institutes of Health (NIH) contributed to every one of the 210 drugs approved by the FDA between 2010 and 2016.<sup>14</sup> Given the sometimes enormous profits that drug manufacturers can make from research funded by taxpayers, manufacturers should be required to disclose research and development costs for drugs, including identifying which portion of research they alone funded versus how much was funded by the NIH, along with research conducted by other academic entities, and/or by another pharmaceutical company (even if it is later acquired by the current manufacturer).

- **Issue Pricing Transparency Reports Based on Pricing Data Submitted by Drug Manufacturers:** A primary reason why the current market for prescription drugs doesn't work is the lack of transparency surrounding drug pricing set by manufacturers. Prices for drugs are clearly rising at rates that far exceed inflation and the level of any rebates or discounts offered by manufacturers to purchasers. CSRxP therefore appreciates that the Consolidated Appropriations Act of 2021 supports drug pricing transparency by requiring HHS to report on its website prescription drug pricing trends and other pricing information based on data submitted by health plans. However, we are concerned about this new data reporting burden on health plans and furthermore believe that the drug pricing data most useful for consumers and taxpayers is the one that derives directly from the original source: the drug industry. The federal government already has data that can demonstrate how changes in list prices are reflected in the prices paid by the Medicare and Medicaid programs. Under the Medicaid Drug Rebate Program, pharmaceutical manufacturers are required to report certain key price terms

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<sup>13</sup> Pollack, Andrew. "[Sales of Sovaldi, New Gilead Hepatitis C Drug, Soar to \\$10.3 Billion.](#)" *New York Times*. February 3, 2015.

<sup>14</sup> Cleary et. al "[Contribution of NIH funding to new drug approvals 2010 – 2016.](#)" *Proceedings of the National Academy of Sciences of the United States of America*. March 6, 2018.

to the Centers for Medicare & Medicaid Services for each of their drugs. These include Average Manufacturer Price (AMP) and Best Price. While maintaining the confidentiality of this data, HHS could provide very useful analysis about drug prices based on the information it already collects from the Medicare and Medicaid programs. HHS should provide any annual report to the public based on manufacturer-reported data that would include, among other items:

1. The top 50 price increases over the last year by a branded drug;
2. The top 50 price increases over the last year by a generic drug;
3. The top 50 drugs by annual spending and how much the government pays in total for these drugs; and
4. Historical price increases for common drugs, including Medicare Part B drugs, over the most recent 10-year period.

Additionally, HHS should continue to maintain the Medicare and Medicaid Drug Dashboards, which provide important information to the public on prescription drug spending in these federal health care programs. The Dashboards should be updated on a regular basis, preferably quarterly but no less than once annually, and include information on list prices, list price increases, year-over-year pricing data, and manufacturer rebates.

- **Scrutinize Direct-to-Consumer Advertising Requirements:** The roughly \$6 billion drug manufacturers annually spend (2016) on direct-to-consumer (DTC) advertising has come under scrutiny as prescription drug spending takes up a larger portion of every health care dollar each year.<sup>15</sup> DTC advertising may potentially lead to overutilization of high-cost drugs, even when highly effective, lower cost drugs are available. Policymakers should assess the impacts of DTC advertising, particularly broadcast advertising, and evaluate the best approaches for conveying key information to consumers, including information on prices, price increases, and clinical effectiveness. At a minimum, all DTC advertising should include list prices and list price increases so that consumers have a more transparent understanding of the actual price of a drug.
- **Limit Third-Party Patient Assistance Schemes Primarily Paid for by Big Pharma that Mask Actual Drug Prices and Raise Costs:** Third-party patient assistance programs can meaningfully help patients afford the often excessively high-priced medications they need to get well and stay healthy. In many cases, however, drug makers simply are funding these third-party assistance programs as a means to shield patients from the actual high prices of these therapies through cost-shifting that only raises overall drug and health plan costs. For example, the House Oversight and Investigations Committee recently found that “[p]atient assistance programs allowed Novartis to reduce patient price sensitivity, and Novartis used its co-payment programs to drive demand, particularly after loss of exclusivity” for the cancer treatment Gleevec.<sup>16</sup> Indeed, internal Novartis documents projected a potential rate of return on the Gleevec co-pay

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<sup>15</sup> Schwartz L and Woloshin S. [“Medical Marketing in the United States, 1997 – 2016.”](#) *JAMA Network*. January 1, 2019.

<sup>16</sup> U.S. House of Representatives House Committee on Oversight and Reform. [“Staff Report: Drug Pricing Investigation Novartis – Gleevec.”](#) October 2020.

assistance program of \$8.90 for every \$1.00 invested at six months prior to loss of market exclusivity.<sup>17</sup> This raises the question of whether these “charitable” assistance programs—which generate a rate of return for pharmaceutical manufacturers—should be claimed as charitable, tax-deductible contributions.

To increase transparency and lower costs, policymakers should require independent third-party patient assistance organizations to report their donors, amounts donated, and the total amount of payment assistance provided to individuals who are prescribed drugs manufactured by any contributor to these organizations. The HHS Office of Inspector General (OIG) should scrutinize third-party patient assistance programs to ensure that such programs do not inappropriately direct patients to certain therapies and do not increase federal spending on healthcare programs. Use of patient assistance programs funded by drug makers should be prohibited in commercial health insurance and the current regulatory ban on use of drug manufacturer assistance coupons in federal health programs should be codified. Co-pay accumulator programs that discourage inappropriate third-party assistance by drug makers should be explicitly permitted in federal health programs and commercial health plans.

**Competition:** The market can work, but right now the market is broken. When companies can raise the price of a drug by 5,000% overnight or increase the prices of old drugs to shadow the prices of comparable new drugs, such practices are signs indicating the prescription drug market is not a healthy, well-functioning one. Given the significant impact of pharmaceuticals and their costs on our health care system, additional elements should be considered, including how best to encourage competition and bring new, lower-cost therapies to patients more quickly. There is no one-size-fits-all solution – what may work best to encourage competition in certain therapeutic areas may not be effective in others. That is why a range of strategies should be advanced, including:

- **Shorten the Exclusivity Period for Biologics and Promote Policies to Increase the Uptake of Biosimilars:** High-cost biologics are one of the primary drivers of increased spending on prescription drugs in Federal health programs and in the commercial market.<sup>18 19 20</sup> Currently, biologics enjoy a 12-year market exclusivity period. Analyses suggest, however, that this amount of time is excessive and prevents lower-cost alternatives from entering the market. Although providing for intellectual property protections is important to encourage innovation and the introduction of medical advancements in the U.S., consideration should be given to shortening the period of exclusivity. It is important to find the right balance of incentives for pharmaceutical companies while alleviating cost pressures for consumers and payers. Action in this area is particularly important, as a growing proportion of the drug development pipeline is comprised of high-cost biologics. At a minimum, if a generous 12-year market exclusivity period

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<sup>17</sup> *Ibid.*

<sup>18</sup> Congressional Budget Office. “[Prices for and Spending on Specialty Drugs in Medicare Part D and Medicaid: An In-Depth Analysis.](#)” March 2019.

<sup>19</sup> HHS Assistant Secretary for Planning and Evaluation. “[Medicare Part B Drugs: Trends in Spending and Utilization, 2006 – 2017.](#)” November 20, 2020.

<sup>20</sup> AARP. “[Rx Price Watch Report: Trends in Retail Prices of Specialty Prescription Drugs Widely Used by Older Americans: 2017 Year-End Update.](#)” June 2019.

is allowed, policymakers must end the gaming of the patent system that now regularly prevents competitors from coming to the market when the exclusivity period expires.

Along these lines, regulatory policies should encourage market entry and uptake of biosimilars in federal health programs like Medicare and Medicaid, as they have significant potential to expand treatment options and reduce costs through increased competition. Likewise, the FDA should take additional steps to promote broader use of biosimilars, for example, by expanding education to healthcare providers, patients and caregivers about the value of biosimilars and interchangeable biologics. The FDA also should revisit the biosimilar “naming” guidance to streamline naming of biosimilar products and encourage appropriate switching to lower cost biosimilars away from expensive brand biologics. The FDA further also should incentivize outcome reporting on patients who switch from brand biologics to biosimilars in clinical trials and post-market surveillance programs.

- **Increase Oversight of Patent Settlements:** Policymakers should encourage robust oversight and opposition to settlements that are deemed anticompetitive and prevent generics and biosimilars from entering the market in a timely manner. These patent dispute settlements result in a generic or biosimilar company agreeing to refrain from marketing its generic or biosimilar product for a specific period of time in return for compensation from the branded company. The Federal Trade Commission (FTC) has cited these arrangements as anti-competitive and estimates that they cost consumers and taxpayers \$3.5 billion in higher drug costs ever year.<sup>21</sup>
- **Target Exclusivity to Truly Innovative Products:** Currently, pharmaceutical manufacturers can extend a patent and market exclusivity protections by seeking approval for a “new” product that is essentially the same as the original product, such as extended-release formulations or combination therapies that simply combine two existing drugs into one pill. Prohibiting such tactics – often referred to as “evergreening” or “product hopping” – will bring generic options and lower costs to consumers more quickly. Pricing schemes that may be deemed anti-competitive should be closely monitored by the appropriate federal agencies including the FTC and prosecuted if found to be in violation of antitrust laws. That is why CSRxP has formally endorsed the Reforming Evergreening and Manipulation that Extends Drug Years (REMEDY) Act in the 116<sup>th</sup> Congress, which targets abusive practices by big pharma to extend patents such anticompetitive “evergreening” practices. Drug manufacturers also should be required to keep their original brand products on the market past the generic entry date to better ensure that more market share moves to the generic product.
- **Apply Stricter Scrutiny to Patent Applications and Thwart Patent Abuse by Curbing Patent “Estates” and “Thickets”:** Biopharmaceutical companies have abused and manipulated the patent laws by creating patent “estates” and “thickets” to extend market exclusivity for their products far beyond the times their original market exclusivity periods have ended. For

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<sup>21</sup> FTC. [“Pay-for-Delay: How Drug Company Pay-Offs Cost Consumers Billions.”](#) January 2010.



example, the blockbuster biologic Humira generated nearly \$20 billion in global sales in 2019.<sup>22</sup> Humira is covered by a total of 247 patents in the U.S. that have the goal of delaying competition with the brand product by 39 years.<sup>23</sup> 89% of those patents were filed after Humira was already on the market and nearly 50% of them were filed after the first patent expired in 2014 and more than 20 years after the initial Humira patents were filed in 1994.<sup>24</sup> Humira's patent "estate" has had significant cost consequences for Medicare and Medicaid, with spending on Humira in these programs increasing by 266% from 2012 to 2016.<sup>25</sup> It has also imposed enormous costs on patients, with average Humira spending per person more than doubling from \$16,000 to \$33,000 between 2012 and 2016.<sup>26</sup> This is why CSRxP supports efforts to thwart patent "thicket" and "estate" abuses by biopharmaceutical companies that have kept exceedingly high-priced drugs on the market for far too long without competition.

In addition to enacting laws that curb anti-competitive and unfair patent "estates" and "thickets," federal regulatory agencies such as the U.S. Patent and Trademark Office (USPTO) should apply stricter scrutiny in patent assessments to determine if patents are overly broad or inaccurate. Standards for patent approval should be raised for those seeking to cover additional uses of existing compounds. Patents should not be approved by the USPTO for changes based on common pharmacological experimentation and knowledge and the USPTO should require demonstration of greater clinical benefit or other higher standards for new formulation patents. Importantly, the USPTO's Patent and Trial Appeals Board (PTAB) *inter partes review* (IPR) process should return to "broadest reasonable interpretation" (BRI) standard, which will promote greater generic and biosimilar competition, and generic and biosimilar innovators should have more time to assess and potentially engage in the IPR process. The PTAB should automatically review all secondary patent when these patents are provided to the FDA in order to limit the number of continuing patent applications that can be filed for the same product. A Patent Quality Taskforce also should be established to make recommendations to Congress to improve the patent system to ensure truly innovative products receive patents. Critically, the USPTO should receive additional funding and resources to carry out this important work that targets abusive intellectual property tactics by brand drug makers to keep their products on the market without competition for far too long.

- **Target Orphan Drug Incentives:** The Orphan Drug Act introduced a range of incentives to encourage the development of medications to treat rare diseases – those that affect fewer than 200,000 individuals and many incentives include waived FDA fees, tax credits, and seven years of marketing exclusivity. Since passage of the Orphan Drug Act, hundreds of orphan drugs have been approved. Many of these medications are helping patients who previously had no other treatment options. However, an increasing number of orphan drugs have achieved blockbuster status, with billions of dollars in sales annually. One factor driving this trend is an increased focus on subcategories of diseases. Drug manufacturers submit FDA applications based on

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<sup>22</sup> Blankenship, Kyle. "[Special Reports: Humira](#)." *Fierce Pharma*. July 27, 2020.

<sup>23</sup> I-MAK. "[Overpatented, Overpriced Special Edition: Humira](#)." October 2020.

<sup>24</sup> *Ibid.*

<sup>25</sup> *Ibid.*

<sup>26</sup> *Ibid.*

these new, considerably smaller populations in order to qualify for orphan drug status. After receiving an orphan drug designation, the pharmaceutical company then seeks additional approvals for non-orphan indications, substantially expanding use of the drug. Utilization can also grow significantly through off-label use.

These dynamics, combined with the high prices orphan drugs command, can lead to blockbuster levels of sales that are clearly inconsistent with a drug receiving an orphan indication.

Policymakers should take steps to ensure that the integrity of the Orphan Drug program is maintained, for example, by requiring that any subsequent orphan indications a product receives after the initial orphan indication to have shorter periods of market exclusivity than the seven years of exclusivity awarded for the first orphan indication. Further, HHS should take steps to assess such trends and ensure that the Orphan Drug Act's incentives are being utilized to develop medicines to treat true rare diseases. For example, the FDA could require additional information when companies seek orphan drug status, such as providing information about additional indications for which a company intends to see approval. HHS also should analyze and report on orphan drug utilization and pricing trends, including trends by indication for orphan and non-orphan uses.

- **Foster Competition by Curbing Citizen Petitions:** Delays in the FDA approval process often prevent competitors from coming to market in a timely manner. For example, although Congress intended the FDA "citizen petition" process to raise valid scientific issues with generic drug applications, in actuality brand drug makers have filed most of citizen petition requests with the primary purpose of delaying FDA approval of generic competitors to their brand drugs. To inappropriately game the system, brand drug makers oftentimes request that FDA mandate additional testing or other requirements; in most cases, FDA denies such requests – but through a relatively lengthy process that still delays generic drug market entry. That is why CSRxP has endorsed the Stop Significant and Time-wasting Abuse Limiting Legitimate Innovation of New Generics (Stop STALLING) Act in the 116<sup>th</sup> Congress, which provides that the submission of a citizen petition to prevent or delay entry of a generic or biosimilar is presumptively illegal under antitrust laws and gives FTC authority to seek civil penalties and other relief in response to filing sham citizen petitions.

In addition, existing FDA regulatory tools should be brought to bear to bring potential market competitors to the market more quickly. Indeed, several FDA programs are intended to facilitate and expedite review of new drugs that address unmet medical needs in the treatment of a serious or life-threatening condition. These include fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation. These incentives should also be utilized to encourage additional market entrants and drive competition for expensive classes of treatments where there are no competitors and to encourage a second or third market entrant. Such a strategy could not only increase competition, but also will serve as an important protection to consumers if the first market entrant has to be withdrawn due to safety concerns.

- Strengthen Post-Market Surveillance:** Today, manufacturers may be required to conduct additional clinical trials post approval of a particular drug or biologic. This is often the case when expedited approval pathways are used, which typically involve smaller clinical trials with a narrower patient population. In such cases a given drug may show significant promise and because of high unmet need, the FDA wants to get the product to market despite an incomplete understanding of long-term efficacy or side effects. However, once a drug is approved, many of these studies are never conducted. A related concern is that for clinical trials to be completed, much of this information is never reported publicly. Specific timelines must be put in place to ensure that post-market trials are conducted. If manufacturers do not follow through with their commitment to complete a required trial, they should be subject to fines or other penalties unless an exception has been granted by the FDA. In addition, manufacturers should be required to report summary data for all trials (whether a product is approved or not) that includes non-identifiable data on demographics and baseline characteristics of participants, primary and secondary outcome results, and information on any adverse events.

**Value:** Investing in the development of information assessing the effectiveness of different treatment options is a critical component to addressing high prices of prescription drugs. As the health care system drives to deliver higher value, there is insufficient evidence as to how new (and often expensive) drugs compare with older interventions. While other countries require data comparing various treatments to help reach a value-based price, the U.S. market allows drug manufacturers to set the price without asking manufacturers to justify the cost. The following efforts should be advanced to bring better information to providers and patients about the value of different treatments:

- Expand Research on Treatment Comparative Effectiveness and Value:** Consumers and providers should be empowered to know which treatments and drug regimens work best and which are less effective. Policymakers should increase funding for private and public efforts aimed at providing information on the comparative effectiveness of different treatments to physicians and their patients, which can help them make appropriate assessments about the value of different treatment approaches, particularly those with very high costs. A prime example is the Institute for Clinical and Economic Review (ICER). ICER is a non-profit organization that evaluates the evidence of the value of medical tests and treatments with an aim toward improving patient care and controlling costs. Another example is the Patient-Centered Outcomes Research Institute (PCORI), which is a government-sponsored, non-profit institute focused on producing research to better inform patients, caregivers, payers, and healthcare providers on the comparative effectiveness of various health care interventions.
- Require Drug Manufacturers to Conduct Comparisons of New Products to Existing Ones:** Many other countries currently require drug manufacturers to provide a dossier of comparative effectiveness research (CER) studies to demonstrate that their product is better than the previously existing standard treatment in terms of clinical or cost/value efficacy. That information is not consistently shared with U.S. policymakers or regulators. Efforts should also be made to improve public access to CER information so consumers and clinicians are able to consider clinical and/or cost-effectiveness information in evaluating clinical intervention options. CER also should be considered by payers, along with other factors, in making evidence-based benefit decisions. Expanding the availability and use of such research among U.S.

consumers, clinicians and payers would foster evidence-based competition and reduce spending on unnecessary or ineffective treatments.

- **Require Innovative Payment and Incentive Structures that Promote Value in Government Health Care Program:** Medicare should take action to engage in new payment models that promote value-based payments for drugs. While several models are being developed in the private sector – including indication-based payments and outcomes-based contracts – government programs have lagged behind. These models can provide enhanced financial incentives for manufacturers of new drugs and medical technologies that are contingent upon agreed-upon standards for quality care and outcomes. Such strategies should be encouraged to the maximum extent possible.