



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. Today, prescription drug expenditures make up nearly 20 percent of all health care costs, and prescription spending is growing faster than any other part of the health care dollar.

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 restore a functioning market by increasing **transparency**, promoting **competition** and innovation, and that result in **value**.

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.¹ There is no way to verify this figure. Additionally, there is no way of determining how much pharmaceutical companies actually invest in research and development (R&D) activities. This missing information could play an extremely important role in assessing whether a drug's price is 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 with price changes for existing drugs and the 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 tossed aside with unfounded claims that any steps toward transparency will erode pharmaceutical investments in R&D.

- **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, the label being discussed with the FDA that indicates the 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 well as how many times a year the price of a drug has been increased. Information would be reported to the Department of Health and Human Services (HHS), with protections in place to exclude sensitive, proprietary information.

¹ Tufts Center for the Study of Drug Development, "Cost to Develop and Win Marketing Approval for a New Drug Is \$2.6 Billion," Press release, November 18, 2014.

Transparency requirements are not without precedent. Currently, many entities in the healthcare sector report data to governmental entities. Health plan issuers are required to provide premium information to state insurance commissioners many months in advance of those premiums being used in the market. In 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. As another example, hospitals, skilled nursing facilities, and certain other providers are required to submit cost report data to HHS annually. This includes information on facility characteristics, utilization data, costs and charges, and financial data. This policy would simply extend transparency to the pharmaceutical sector as well.

- **Ensuring 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 Sovaldi was largely conducted by a small bio-tech company (later acquired by the current manufacturer) that received the majority of its funding from the National Institutes of Health (NIH). Gilead purchased this bio-tech, and recouped the cost of the acquisition in one year of sales of the \$1,000 per pill drug.

Manufacturers should be required to disclose research and development costs for drugs, including the portion of research funded by the manufacturer versus research funded by NIH; research by other academic entities; or research conducted by another pharmaceutical company (later acquired by the current manufacturer).

- **Pricing Transparency Reports:** A primary reason why the current "market" for drugs doesn't work is the lack of transparency surrounding drug pricing. Prices for drugs are clearly rising at rates that far exceed inflation and the level of any rebates or discounts offered by manufacturers. The federal government 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 Rebate Program, pharmaceutical manufacturers are required to report certain key price terms to the Centers for Medicare & Medicaid Services (CMS) 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 an annual report to the public which 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.

- **Continued Assessment of Direct-to-Consumer Advertising Requirements:** Direct-to-consumer (DTC) advertising has come under scrutiny as prescription drug spending takes up a bigger portion of health care dollars each year. There is concern that DTC advertising has the potential to lead to over utilization 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.

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 when the prices of old drugs rise to shadow the prices of comparable new drugs, that is not a healthy, functioning market. Given the significant impact of pharmaceuticals 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 the best solution in other areas. That is why a range of strategies should be advanced:

- **Reduce Backlog of Generic Applications:** The FDA continues to face a significant backlog of generic drug applications, with nearly 4,000 applications pending and average approval times reaching three or more years. The FDA should be provided the resources necessary to clear this backlog and prioritize approval of generic drug applications, particularly for classes of drugs with no or limited generic competition, including necessary funding, personnel, and/or programs to speed the review of generic drugs in shortage or single source generic drugs.
- **Foster Competition for Branded Drugs:** Delays in the FDA approval process often prevent competitors from coming to market in a timely manner. In such situations, adjustments should be made to bring potential market competitors to the market more quickly. The tools to do so already exist. For example, 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.
- **Curb Misuse of REMS:** The FDA requires manufacturers to submit structured plans, known as Risk Evaluation and Mitigation Strategies (REMS), for drugs associated with known or potential risks that may outweigh the drugs' benefits. For example, REMS may require sponsors to create additional safety information for patients or to ensure that health care providers that prescribe certain drugs have special certification. Of concern, however, is when brand-name manufacturers use REMS strategies, under the guise of

patient safety concerns, to block generic manufacturers from obtaining samples of brand drugs needed for bioequivalence testing and FDA approval. Steps should be taken to ensure that REMS are not inappropriately used to stifle the introduction of generic competition and lower cost options for consumers and payers.

- **Strengthen Post-Market Surveillance:** Today, manufacturers may be required to conduct additional clinical trials post approval of a particular product. 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 the clinical trials that are 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 summarizes non-identifiable data on demographics and baseline characteristics of participants, primary and secondary outcome results, and information on any adverse events.
- **Target Exclusivity Protections to Truly Innovative Products:** Currently, pharmaceutical manufacturers can extend 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 anticompetitive should be closely monitored by the appropriate federal agencies and prosecuted if found to be in violation of antitrust laws.
- **Increase Oversight of "Pay for Delay" Settlements:** Policymakers should encourage robust oversight and opposition to settlements that are deemed anticompetitive and prevent generics from entering the market in a timely manner. These patent dispute settlements – often referred to as "pay for delay" – result in a generic company agreeing to refrain from marketing its generic product for a specific period of time in return for compensation (often undisclosed) 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 every year.
- **Shorten the Exclusivity Period for Biologics and Promote Policies to Increase the Uptake of Biosimilars:** Currently, biologics enjoy a 12 year market exclusivity period. Analyses suggest this amount of time may be unnecessary 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. market, consideration should be given to shortening the periods

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. Similarly, regulatory policies should encourage market entry and uptake of biosimilars, as they have significant potential to expand treatment options and reduce costs through increased competition.

- **Targeted Orphan Drug Incentives:** The Orphan Drug Act introduced a range of incentives to encourage the development of medications to treat rare diseases -- diseases that affect fewer than 200,000 individuals. These 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 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 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. 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 seek approval. HHS should also analyze and report on orphan drug utilization and pricing trends, including trends by indication for orphan and non-orphan uses.

Value: Investing in the development of information that assesses the effectiveness of different treatment options is a critical component to addressing the high price 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 Effectiveness and Value:** Consumers and providers should be empowered to know which treatments and drug regimens work 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 on the value of medical tests and treatments with an aim toward improving patient care and controlling costs. Recently, ICER released an important draft report on PCSK9 inhibitors for treatment of high cholesterol. In its draft assessment, ICER concluded that the price that best represents the overall benefits of these new drugs would be between \$3,600 and \$4,800 – a 67% discount off the manufacturers' list price of about \$14,000 per year of treatment. Investments in the development of information such as this are critical for physicians, patients, and payers as more and more high cost drugs are introduced into the health care system.

- **Drug Manufacturers Should be Required to Conduct Comparisons of New Products to Existing Products:** 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. Efforts should also be made to improve access to CER information so consumers and clinicians are able to consider clinical and cost-effectiveness information in evaluating clinical intervention. CER should also be considered by payers, along with other factors, in evidence-based benefits. Expanding the availability and use of such research among U.S. consumers, clinicians, and payers would help create evidence-based competition and reduce spending on unnecessary or ineffective treatments.
- **Expand the Availability of Value-Based Pricing:** Most federal health programs cover prescription drugs for their beneficiaries. Steps should be taken to ensure these programs, such as Medicaid and Medicare, can best take advantage of recent innovations in value-based purchasing, including identification of necessary modifications to ensure all parts of the U.S. health care system can benefit from market-based negotiating efforts to lower drug prices.
- **Government Programs Should Require Innovative Payment and Incentive Structures that Promote Value:** Medicare should take action to engage in new payment models that promote value-based payments for drugs. While several models are starting to be 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.