Promoting Value, Affordability, and Innovation in Cancer Drug Treatment

A Report to the President of the United States from the President's Cancer Panel

Executive Summary

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Innovations in cancer therapy, particularly the development of targeted drugs and immunotherapies, hold remarkable potential to transform treatment of the disease. Increasingly, a new generation of cancer drugs is producing durable remissions and, potentially, cures. However, prices for these drugs have risen dramatically in recent years. The United States faces the challenge and tension of creating both a robust pipeline of innovative cancer drugs while ensuring that these drugs are accessible and affordable for those who need them. For its 2016–2017 series of workshops, the President's Cancer Panel examined the drivers and impact of rising cancer drug prices in the United States and developed recommendations to address this problem.

The Panel concluded that urgent action is needed to address the ongoing, rapid increases in cancer drug costs—the health and lives of patients are at stake. This challenge can only be met through the input and action of all stakeholders—drug developers and manufacturers, policy makers, government, public and private payers, healthcare institutions and systems, providers, and patients.

Actions to address drug costs should follow several key guiding principles—**cancer drug prices should be aligned with their value to patients, all patients should have affordable access to appropriate cancer drugs, and investments in science are essential to drive future innovation**. Collectively, these actions will help us reach the ultimate goal of ensuring that all patients receive the treatment they need and experience the benefits that these remarkable drugs can offer.

PART 1: THE RISING COST OF CANCER DRUGS: IMPACT ON PATIENTS AND SOCIETY

The recent, dramatic rise in drug prices is straining patient, health system, and societal resources. Drugs account for about 20 percent of the total costs of cancer care in the United States, but cancer drug costs are accelerating faster than costs for other components of care. Launch prices of cancer drugs in the United States have risen so steeply over the past few decades that they have quickly outpaced growth in household incomes. U.S. patients and their insurers are paying more than ever for cancer drugs—$54,100 for a year of life in 1995 compared with $207,000 in 2013. Unfortunately, there are no signs that this price escalation is slowing.

The burden of high drug costs on patients—even those with health insurance—can be significant. Out-of-pocket spending on drugs can be hundreds, or even thousands, of dollars a month for patients in active treatment. Patients with higher out-of-pocket expenses are less likely to adhere to recommended treatment regimens, which may have a detrimental impact on outcomes. Although out-of-pocket expenses for drugs can be high, they are only one of many costs cancer patients face. The term **financial toxicity** describes the negative impact of cancer care costs on patients' well-being. Like medical toxicities caused by cancer treatment, financial toxicity can impose a significant burden on patients, including a diminished quality of life, interference with high-quality care delivery, and even a reduction in survival rates.

PART 2: TAKING ACTION TO PROMOTE VALUE, AFFORDABILITY, AND INNOVATION IN CANCER DRUG TREATMENT

Some cancer drugs have been transformative—significantly improving patients' outcomes and, in some cases, producing long-term remissions. However, many new drugs do not provide benefits commensurate with their prices. The Panel concluded that misalignment of drug prices and value is a critical problem that must be addressed. High-value drugs that cure cancer, significantly extend survival, and/or substantially improve quality of life should be priced higher than drugs that provide only modest benefits. They must be priced, however, within reach of the patients who need them. In this report, the Panel makes several recommendations to maximize value and affordability while continuing to support a pipeline of biopharmaceutical innovation. The ultimate goal is to ensure that all cancer patients—now and in the future—have affordable access to high-value drugs without experiencing financial toxicity.

While the focus of this report is on cancer drug costs and access, the Panel recognizes that rising cancer care costs overall also are a serious concern. Efforts to address cancer drug costs should be undertaken with consideration of the total cost of cancer care.

Recommendation 1. Promote value-based pricing and use of cancer drugs.

Steps must be taken to better align drug prices and costs with their value and promote use of high-value drugs. Achieving these goals could improve the quality of cancer care; create incentives for development of innovative, effective new drugs; and help address increases in drug spending that are threatening to put high-value drugs out of reach for some patients.

A Value Framework Is Needed to Facilitate Value-Based Pricing

There is no broadly accepted framework in the United States for determining whether cancer drug prices are aligned with their value. Defining the value of cancer drugs is challenging. Numerous factors influence value, and the relative importance of each of these factors depends on the perspective of the stakeholders—patients, providers, payers, healthcare systems, manufacturers, researchers, and society. Despite these challenges, cost can no longer be ignored if the United States aims to balance a robust innovation pipeline with treatment that is accessible and affordable for all cancer patients.

Developing and implementing a widely accepted value framework for cancer drugs is a critical step toward value-based pricing. Taking this step will require input and collaboration from all involved stakeholders, understanding that patient benefit must be central when assessing value. An ideal framework would integrate information on clinical outcomes, toxicities, impact on quality of life, and costs. It would inform negotiations between drug manufacturers and payers and also could guide development of value-based payment models and benefit designs that promote selection of high-value drugs by physicians and patients. Value assessments also could inform shared decision making among patients and providers and potentially improve patient outcomes.

Outcomes-Based Pricing for Cancer Drugs Should Be Explored

Outcomes-based risk-sharing agreements link payment for a drug to patients' outcomes. Under these agreements between payers and manufacturers, manufacturers are not paid or are paid less when patients do not achieve established clinical and/or quality-of-life outcomes. Although linking price to outcome does not guarantee value-based prices, outcomes-based pricing has potential to improve

alignment of drug price and value. More research is needed to determine the impact of outcomes-based pricing on value, quality, and costs for patients, providers, and payers, as well as the most effective and efficient ways to structure these agreements in various situations. Public and private payers and manufacturers should develop and pilot-test outcomes-based risk-sharing agreements for cancer drugs.

Payment Models Should Incentivize Providers to Use High-Value Drugs

The ways in which providers and healthcare organizations are paid influence choices about healthcare and how care is delivered. Under the prevailing fee-for-service payment model in the United States, providers are reimbursed largely based on the individual services and products they deliver. Current payment policies may create incentives for providers to deliver more services, prescribe more drugs, and/or prescribe higher-priced drugs. Physicians and hospital systems should be incentivized to recommend the highest-value treatment based on patients' clinical presentation and preferences, free of financial incentives to use higher-priced options. Ongoing healthcare reform efforts in the United States include alternative payment models that reward providers for providing high-quality, cost-efficient care rather than reimbursing them based solely on the volume of services delivered. Public and private payers should develop and test alternative payment models that support delivery of high-quality cancer care, including high-value drugs.

Insurance Plans Should Promote Patients' Use of High-Value Drugs

As drug costs have increased in recent years, many insurance plans have established drug tiers with different cost-sharing structures (patient out-of-pocket requirements) to steer beneficiaries toward preferred drugs. Value-based insurance design (VBID) offers a more patient-centered approach to insurance benefit design by aligning patients' out-of-pocket costs with the *value*—not the costs—of drugs and services. VBID may be well suited to cancer care due to the increasing role of high-cost specialty drugs and the growing capability to use biomarkers to match drugs with patients most likely to benefit. Public and private payers should develop and test VBID programs that promote patients' use of high-value cancer drugs.

Recommendation 2. Enable meaningful communication about treatment options, including cost information, to support patients' decision making.

After discussion with their cancer care teams, patients should be empowered to select treatments aligned with their needs, values, and preferences. To accomplish this, they must have accurate information about their disease, clear understanding of treatment options, and access to information about costs of treatment options. Cancer care teams should tailor this information to the needs, preferences, and comprehension capacity of individual patients.

Cancer patients express interest in communicating with their healthcare providers about cost, though such discussions are infrequent—only 27 percent of cancer patients and less than half of oncologists surveyed reported having had cost-related discussions. Research is needed to identify the best ways to communicate about cost and help patients include cost in their assessments of treatment value. It will be important to determine how cost discussions affect clinical decision making and clinical outcomes, as well as patients' quality-of-life, well-being, satisfaction, and financial toxicity.

Lack of transparency often makes it difficult for patients to know how much they will be charged for their care and the portion they will be responsible to pay out of pocket. The Panel urges payers and health systems to make cost and price information more widely available to patients and cancer care teams to facilitate informed decision making.

To enable value assessment of treatment options, cost information should be considered in conjunction with potential clinical benefits and harms, including impact on patients' quality of life. However, these data often are limited or unavailable. Physicians should clearly explain any evidence gaps to patients and should also tell patients when a drug is unlikely to provide benefit. In addition, health information technology should be leveraged to address these knowledge gaps.

Recommendation 3. Minimize the contributions of drug costs to financial toxicity for cancer patients and their families.

Patients' out-of-pocket costs for cancer drugs vary widely depending on a number of factors, such as cancer type, treatment plan, treatment setting, insurance status, and benefit design. High out-of-pocket drug expenses can have a detrimental impact on patients' care and well-being. Patients may decide not to fill their prescriptions, skip doses, or take less drug than prescribed to save money. Other patients may deplete their savings, incur debt, or forego spending on necessities to pay for their drugs. Steps should be taken to minimize the contributions of drug costs to financial toxicity for cancer patients and their families.

Health insurance—including prescription drug coverage—is a key factor in ensuring that drugs are affordable for cancer patients. As health insurance access has expanded, fewer Americans—including those with a history of cancer—report foregoing needed drugs because of cost. Future health policies should support and expand, not undermine, this progress. All Americans should have the opportunity to purchase reasonably priced, high-quality health insurance with prescription drug coverage to facilitate affordable access to cancer drugs.

As drug prices have increased, payers have shifted costs to patients through various cost-sharing mechanisms. Cost-sharing is an appropriate way to encourage judicious use of healthcare services, but it should not interfere with access to appropriate treatment or cause significant financial hardship. To protect people from excessive out-of-pocket costs, all public and private insurance plans should include out-of-pocket spending limits.

Recommendation 4. Stimulate and maintain competition in the generic and biosimilar cancer drug markets.

The United States incentivizes innovation, in part by granting patents and a number of exclusivities to manufacturers of new drugs and biologics. Once exclusivity ends, generic drugs and biosimilars can be approved, creating potential for competition and possibly driving down prices. Efforts must be made to facilitate timely and efficient market entry of generic and biosimilar drugs for cancer to bolster competition and ensure affordable access for patients.

The generic drug market has provided patients with affordable access to many drugs. In some cases, however, market forces or anticompetitive behaviors limit competition, which can lead to higher prices and/or drug shortages. The U.S. Food and Drug Administration (FDA) should reduce barriers for generic manufacturers to enter markets with no generic options or too few generic options to create competition. In addition, U.S. regulatory agencies and policy makers should continue to monitor and evaluate the generic drug market to identify factors that prevent healthy competition. Deliberate efforts to limit competition must be addressed. FDA also should continue to monitor the emerging U.S. biosimilars landscape and ensure that approval processes and manufacturing oversight are functioning efficiently such that biosimilar products can be made available to the American public.

Recommendation 5. Ensure that the FDA has appropriate resources to assess cancer drug safety and efficacy efficiently.

FDA plays a critical role in ensuring patient access to innovative cancer drugs. Cancer drug development

and evaluation present distinct challenges, particularly in the age of precision medicine. FDA has implemented policies and programs to address many of these challenges, and the Oncology Center of Excellence was established to enable more efficient and effective review of cancer treatments. The Panel supports the efforts of the Center.

An adequately staffed and well-resourced FDA is more important than ever in the modern era of oncology product development. A highly skilled FDA workforce also is essential as the agency considers important questions about incorporation of new kinds of data, including real-world evidence, into its review processes. The Panel urges the President and Congress to ensure that FDA has the resources and authority to assess the safety and efficacy of oncology products and to appropriately staff the Oncology Center of Excellence.

Recommendation 6. Invest in biomedical research to create a strong foundation for developing innovative, high-value cancer drugs.

A strong research infrastructure and workforce are essential to develop and deploy innovative, high-value drugs that potentially cure or, if not cure, significantly extend and improve the lives of cancer patients. The United States has long been a leader in biomedical research and pharmaceutical innovation, in large part because of cross-sector investment by government, industry, and nonprofit organizations. A vibrant discovery ecosystem is essential to ensure that the cancer drug pipeline continues to produce high-value products that benefit all patients.

The National Institutes of Health (NIH) is the world's leading funding organization for biomedical research. The work carried out by NIH-supported investigators has helped elucidate the molecular underpinnings of several cancer types and contributed to development of novel therapies. NIH training grants and career development programs play a critical role in building the U.S. biomedical research workforce. The Panel urges the President and Congress to provide sustained, predictable funding for NIH that, at a minimum, keeps pace with inflation. Failure to invest in NIH will threaten the United States' role as a global leader in the biomedical sciences and future progress against cancer. The Panel also urges continued commitment to cancer research by other sectors, including nonprofit and philanthropic organizations, venture capital companies, and the biopharmaceutical industry.

PART 3: CONCLUSIONS

Rising cancer drug costs are a significant problem and cannot be ignored—the consequences for patients, families, and society are too great. More than ever, affordable access to drugs will be the difference between life and death for cancer patients. The following principles should guide action:

- Cancer drug prices should be aligned with value to patients.
- All patients should have affordable access to appropriate cancer drugs.
- Investments in science are essential to drive future innovation.

This complex problem will not be solved quickly or easily, and it will not be solved by any organization or sector working alone. The Panel urges all stakeholders—drug developers and manufacturers, policy makers, government, public and private payers, healthcare institutions and systems, providers, and patients—to work together to address rising costs and ensure that patients have access to innovative, high-value, and affordable cancer drugs. The ultimate goal is to ensure that patients receive high-quality cancer treatment and experience the best possible health outcomes without financial toxicity.