

Policy Solutions: Delivering Innovative Treatments to Patients

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Over the last half-century, new medicines, technologies and biopharmaceutical research have revolutionized health care and helped millions of patients live longer, healthier lives. Building on this progress, America now is on the cusp of a golden era of biopharmaceutical discovery and personalized medicine.

At the same time, our health system is evolving to embrace a strong commercial market, value-driven payment and greater patient engagement in health care decision-making. As the health care market continues to evolve, we need to make sure that patient needs and preferences are central.

We are committed to pragmatic, patient-centric approaches to a stronger U.S. health care market, including the biomedical innovation ecosystem. Addressing health care costs holistically, we can build a sustainable, science-based health care system that stems the growth of chronic disease and harnesses today's hopes to discover tomorrow's cures.

Our specific proposals for improving the health care system and sustaining affordability include:

Modernizing Drug Discovery, Development and Approval

Pro-patient, pro-science, pro-market reforms at the Food and Drug Administration would enhance the competitive market for biopharmaceuticals, drive greater efficiency in drug development and discovery and help hold down costs for payers and consumers.



Promoting Value-Driven Health Care

Payers face challenges in paying for the value of prescription medicines because regulatory barriers impede open communication by manufacturers, predictability regarding the biopharmaceutical pipeline and innovative contracting. Value-driven payment for prescription medicines can promote efficiency and affordability by ensuring that more patients receive the best treatment for them, the first time around. We need to remove the hurdles making it difficult for innovative, sensible payer/manufacture arrangements to emerge.



Engaging and Empowering Consumers

A well-informed consumer is an engaged and empowered patient. We need to make more information on health care out-of-pocket costs and quality available to patients. In addition, vulnerable patients should have the protection of enforceable, common sense rules that prevent discrimination and remove barriers to access. These steps will improve coverage and access and help make medicines more affordable to patients.



Addressing Market Distortions

The market-based U.S. health care system has worked well over time, but more can be done to help the market work even better. Addressing distortions – like the rapidly growing 340B program or the risk adjuster for commercial insurance that does not account for prescription drug costs – would help preserve the safety net, revive the health care market and improve affordable access to medicines for patients.



Developing an innovative medicine is a lengthy and complex process, taking an average of 10 or more years. The clinical trial component alone takes roughly six to seven years. With just 12 percent of drugs that enter clinical trials resulting in an approved medicine, the average research and development cost for each successful drug is estimated at \$2.6 billion (including the cost of failures). Against this backdrop, pro-patient, pro-science, pro-market reforms at the Food and Drug Administration (FDA) would enhance the competitive market for biopharmaceuticals, drive efficiency in drug development and discovery and help hold down costs.

SOLUTION #1

Encourage Use of 21st Century Tools for Drug Evaluation, Review and Approval

Scientific advances are re-shaping our understanding of the causes of disease, creating new avenues of research, exploration and discovery. New and powerful tools emphasize individual patient characteristics and include innovative clinical trial design, advanced statistical methods and use of real-world evidence.

Example #1: Biomarkers hold promise for improving and accelerating drug development. A biomarker is an objective measure of normal biologic processes, pathologic processes or biological responses to a therapeutic intervention. Broader use of biomarkers may speed and improve the quality of evidence development to support medical product evaluation and may accelerate product development timelines. For example, idiopathic pulmonary fibrosis (IPF) is a serious and life-threatening disease of the lung that involves progressive scarring leading to loss of lung function and ultimately death. Its cause is unknown. In late 2015, two new drugs were approved for treatment of IPF based on a biomarker-based measurement of delay in decline in lung function. Relying on the biomarker instead of mortality-based assessment allowed much earlier consideration of the drugs' efficacy by the FDA, leading to speedier approvals and access for patients with this difficult disease. In addition to biomarkers, patient-reported outcomes could help enhance FDA decision-making by integrating the patient's perspective into benefit-risk evaluations.

Example #2: New approaches to clinical trial design and statistical methods that leverage scientific advances could lead to greater efficiencies in drug development that could reduce the cost and time to bring a new medicine to market. The randomized, controlled clinical trial design needed for FDA approval has become increasingly complex over time, and regulatory acceptance of innovative approaches has not kept pace with advances in the field. The use of adaptive designs and advanced statistical methods, for example, would increase clinical trial efficiency. Appropriate use of real-world evidence also would allow information other than that derived from traditional studies to aid regulatory decision-making.

Solution: We need to modernize the FDA to keep pace with scientific discovery, including catalyzing the agency's acceptance of innovative drug development tools and real-world evidence to drive greater efficiency. This will yield a more competitive, innovative and sustainable biopharmaceutical ecosystem that better reflects patient experience and perspective.



SOLUTION #2

Ensure FDA Drug Approval Is Scientifically Sound and Efficient

Medical and basic science is advancing at a breathtaking rate. New developments – including those in immunologic and cell therapies, personalized medicine and regenerative medicine – hold the promise of treating debilitating diseases such as Alzheimer's, cancer, diabetes and many rare disorders.

Example #1: For the FDA to keep up with the rapid pace of scientific advances, it must be able to deploy the most modern technologies and access the brightest minds to review cutting-edge scientific developments. Ensuring that FDA's drug review staff infrastructure is strong will avoid needless delays in drug review and approval that lead to longer development times, missed opportunities, higher drug development costs and delays in treatments reaching patients.

Solution: FDA must be allowed to add internal expertise by granting the agency sufficient and efficient hiring authority to attract and retain a strong scientific workforce, including biostatisticians, pharmacologists and geneticists. FDA should also be allowed to pilot new ways to access external expertise through collaborative partnerships with academia and the National Institutes of Health.

SOLUTION #3

Reduce the Generic Backlog and Incentivize Competition Where Needed

A generic drug enters the market at the end of an innovative medication's lifecycle. When it does, the FDA allows the manufacturer to submit an Abbreviated New Drug Application (ANDA), which does not require repetition of time consuming and costly clinical trials the innovative biopharmaceutical company conducted. As a result, generics can enter the market at a fraction of the price of an innovator medicine. With nearly 90 percent of all U.S. retail prescriptions filled with generics, their timely approval is critical to patient access and the long-term sustainability of our health care system.

Example #1: Due to a significant backlog of ANDAs at the FDA, on average it currently takes over four years for the FDA to act on a single application.

Solution: To speed access to generic drugs, the next reauthorization of the Generic Drug User Fee Act (GDUFA) should consider additional steps to improve ANDA review efficiency. In this way, the current backlog of pending applications and the average time required to review generic drug applications can be reduced.

Example #2: For serious diseases or conditions in small patient populations, lack of availability of effective medicines with no remaining patent life or regulatory exclusivity, coupled with no or limited brand or generic competition, may constitute an important public health risk.

Solutions: We need to explore opportunities to encourage competition and catalyze generic entry when the market demonstrates a need. One approach might be to incentivize new sources of older, off-patent medicines for unmet medical needs with appropriate safeguards to prevent abuse. Financial incentives could encourage generic entry, such as an expanded tax credit for the development and manufacturing of generic drugs or a targeted grant program to support generic manufacturing investments and maintain production for eligible products. Another solution may be to provide regulatory incentives so more than one generic drug manufacturer enters the market for treating serious conditions.

Payers face challenges in paying for the value of prescription medicines because regulatory barriers impede open communication by manufacturers, predictability regarding the biopharmaceutical pipeline and innovative contracting. Value-driven payment for prescription medicines can promote efficiency and affordability by ensuring that more patients receive the best treatment for them, the first time around. We need to remove the hurdles making it difficult for innovative, sensible payer/manufacturer arrangements to emerge.

SOLUTION

#1

Improve Predictability for Payers and Diffusion of Evidence

In recent years, payers have expressed a need for greater predictability and certainty regarding the biopharmaceutical pipeline as they often set premiums and formularies 18 months in advance. Yet outdated Food and Drug Administration (FDA) regulations chill manufacturer/payer discussions. Further, once a medicine is approved, current rules can also prevent timely dissemination of important safety, value or efficacy information about a product when the information is not already included in the product's labeling.

Example #1: Under current policy, manufacturers are constrained in sharing safety and efficacy information about medicines in development with payers, health systems and health care professionals. Until a medicine is approved, there is no safe harbor to share proactively such important information as data on expected indications and the expected effect on the patient population. The ability to share this information earlier would give payers a better sense of potential costs and help them set premiums accordingly.

Solution: The FDA should update its regulations to allow manufacturers to share appropriate, scientifically sound information with payers pre-approval to reduce uncertainty and improve payer ability to set premiums.

Example #2: Under current FDA policy, manufacturers conducting research and publishing peer-reviewed studies showing a medicine can help reduce hospital admissions may not proactively share this information, even with payers or providers at financial risk for hospital readmissions. They may be unable to proactively share results of a clinical trial showing a medicine dramatically reduces prevalence of another disease even where the trial was required by the FDA. And current FDA guidance does not allow companies to share peer-reviewed medical literature if the data are not reflected in the labeling and the study is authored by the company – even if FDA relies on the study in its approval decision. As a result, important medical findings may not be widely recognized, or changes in treatment could be delayed by several years while new data are added to the labeling.

Solution: The FDA should update its regulations to allow manufacturers to proactively share truthful, non-misleading information on clinical and economic outcomes with payers and providers after approval.



SOLUTION #2

Address Regulatory and Legal Uncertainty Around Value-Based Payment Arrangements

As we move toward value-driven health care, the limits of regulations developed for a fee-for-service world become clear. Biopharmaceutical companies are exploring a wide range of innovative partnerships with private payers and providers that recognize improvements in care and better outcomes for patients. But significant regulatory and legal uncertainty is slowing the development of sensible new business models.

Example: Despite extensive research demonstrating that improved adherence reduces medical spending for chronic diseases, broadly written laws designed to prevent fraud in Medicare and Medicaid also may preclude certain adherence support arrangements designed to help patients.

Further, biopharmaceutical manufacturers must adhere to a complex set of government price-reporting rules for calculating Average Sales Price in Medicare Part B and Best Price in Medicaid. These highly technical price-reporting rules were not established with new approaches to contracting in mind (such as indication-based pricing or outcomes-based arrangements). While the price-reporting rules do permit manufacturers to make reasonable assumptions, ambiguity about how to capture innovative pricing methods in an Average Sales Price or Best Price framework can create significant uncertainty for manufacturers and payers.

Solution: The Department of Health and Human Services should update regulations or guidance to allow new business models in the private market to evolve, including clarification of fraud laws to support better use of medicines through patient adherence programs; and address any outstanding questions related to price reporting as requested by manufacturers. Changes to regulations or new guidance should be developed through notice and comment.

SOLUTION #3

Improve Patient-Centered Decision Support and Value Frameworks

We face a pressing need to develop rigorous, scientifically sound decision-support tools and definitions of value that reflect the full range of stakeholder perspectives. Sound, well-structured tools for value assessment can help us move beyond silos to a more holistic perspective on value that is central to long-term solutions and patient needs. Better information can help empower informed patient-physician decision-making and make sure patients have a say in the direction of their care.

Example: Organizations, such as the American Society for Clinical Oncology (ASCO) and National Comprehensive Cancer Network (NCCN) have recognized the need for new value frameworks to support patient-physician decision-making, and highlight the importance of accommodating patient differences within these frameworks. But other frameworks, such as the one developed by the Institute for Clinical and Economic Review (ICER), move in the opposite direction by focusing on payers' short-term budget needs and failing to adequately account for individual differences among patients with a one-size-fits-all standard.

Solution: We need to focus on delivering the right medicine, to the right patient, at the right time. Value frameworks and assessments should be developed through an open and transparent process; include meaningful input from patients and providers; support patient differences and preferences; and deliver reliable and relevant information to inform health care decision-making. These tools should be used in ways that help physicians and patients decide which treatment options fit an individual best, and not in ways that replace personalized treatment decisions.

Informed, engaged consumers are well equipped to judge the value of their health care. And yet, consumers too often are frustrated by hurdles that make it hard to access information about their insurance benefits and out-of-pocket health care costs. By removing some of these hurdles, we can help consumers play a larger role in shaping a sustainable, market-based health care system.

SOLUTION

#1

Improve Access to Important Out-of-Pocket Cost Information

Today's health care consumers are more likely to be insured than ever before, but too many still have difficulty accessing the medicines they need. One of the biggest hurdles is high out-of-pocket costs. In an effort to keep premiums low, insurers are increasingly using higher deductibles and other cost sharing that is passed on to the consumer.

Example: When shopping for an insurance plan in a health insurance exchange, it is virtually impossible for consumers to predict their out-of-pocket costs for specific treatments or services. Because a consumer cannot calculate estimated out-of-pocket costs for the medicines they need before picking a plan, it is especially difficult to figure out which plan meets their needs and fits their budget.

Solution: Consumers should have more information about coverage and total costs, including premiums and potential cost sharing, prior to enrolling in any insurance plan. At a minimum, the federal exchange should improve its out-of-pocket costs calculator to allow more personalized estimates. Better-informed coverage choices may lead to higher satisfaction, better health, slower premium growth and lower out-of-pocket costs.

SOLUTION

#2

Improve Access to Important Quality Information

A range of private entities now evaluate the quality of health insurance plans, yet this information is not relayed in an easily digestible format to consumers.

Example: Health insurance exchange plans do not have easily accessible information on quality available, including how other consumers rate a plan or other forms of evaluation. This information is important to both regulators and consumers to ensure plans meet quality standards.

Solution: Additional disclosure to consumers would raise the bar on health plans and help discourage practices that only benefit insurers. Health insurance exchanges should follow Medicare's lead and make simple, transparent health plan quality ratings available. When insurers are required to disclose key information about their interactions with patients and providers (e.g., consumer and provider complaints; coverage denials and reversals; and clinical bases for utilization management, coverage exclusions and care protocols) consumers can better evaluate their coverage choices – and insurers have an incentive to eliminate practices that leave customers dissatisfied.



SOLUTION #3

Improve Access to Important Clinical Information

Insurers apply a range of techniques that impact clinical care and patient choices. These practices are directed by insurers and may interfere with a physician's preferred course of treatment for an individual patient.

Example: Health insurance plans use techniques such as prior authorization, utilization management, preferred clinical pathways or protocols and more. These often come with financial incentives for physicians to comply. When different payers use undisclosed and/or variable clinical evidence to support these policies, consumers may be unable to consider their impact when choosing among services, providers or coverage.

Solution: Insurers should be transparent and held accountable for the clinical criteria they use to incentivize or discourage use of specific services and treatments.

SOLUTION #4

Remove Potential Discrimination in Insurance Benefit Design

Access to prescription medicines is essential to successful treatment of a range of conditions, including cancer, diabetes, HIV/AIDS and mental illness. Yet in some cases, patients with certain conditions have no preferred therapy options available. This can discourage enrollment by individuals with chronic conditions and is in direct conflict with prohibitions against discriminatory benefit design.

Example: In health insurance exchange plans, some insurers are placing all options for medicines to treat a condition on the top cost-sharing tier. In an analysis of 2015 silver exchange plans, five classes of medicines had all drugs placed on the top cost-sharing tier, including generic treatment options.

Solution: State and federal regulators should prohibit plans from structuring formularies in a discriminatory fashion and should carefully scrutinize the use of utilization management tools that might have the same effect.

A well-functioning market can ensure patient access to innovative medicines without sacrificing investment in future treatments and cures. The market-based U.S. health care system has worked well over time, but more can be done to help it work even better. Addressing distortions would help improve affordable access to medicines for patients, protect the safety net and revive the U.S. health care market.

SOLUTION

#1

Address Market Distorting Price Controls

Price controls and government-mandated discounts exist in the United States in several contexts. By holding prices for prescriptions artificially low, this approach can lead to cost shifting within the market and/or reductions in private sector research and medical discovery.

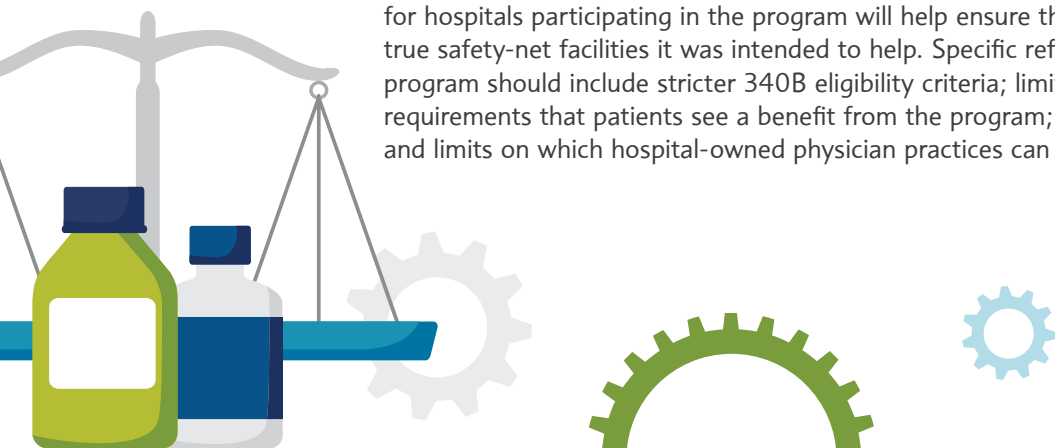
Example: The 340B program currently accounts for roughly 6 percent of U.S. branded drug sales (non-Medicaid) and is valued at roughly \$18.5 billion. These figures are projected to increase to 8 percent and \$25.1 billion by 2019. The overall numbers mask the concentration of 340B in certain therapeutic classes. In cancer classes, for example, 340B hospitals account for 62 percent of hospital reimbursement.

Congress created 340B in 1992 to help vulnerable or uninsured patients access medicines at safety-net facilities. Manufacturers provide steep, mandatory discounts on medicines to eligible facilities. Even as the number of Americans with insurance is on the rise, however, 340B is growing exponentially. This is in part due to eligibility criteria for hospitals that rely in part on the number of Medicaid patients a hospital treats. Accordingly, Medicaid expansion has increased eligibility for 340B even as hospitals' uncompensated care burden is declining. Today, roughly 45 percent of all Medicare acute care hospitals participate in 340B.

Many hospitals have further expanded their reach by buying community-based physician practices and/or through contractual profit-sharing arrangements with pharmacy chains. While clinics participating in the 340B program have requirements on demonstrating patient benefit, there is no similar requirement for hospitals and their affiliates, including retail pharmacy chains that profit from the program.

In too many cases, 340B discounts are becoming a windfall for hospitals, a trend that will continue to distort the market and apply upward pressure on pricing for other payers. According to a recent article in the *New England Journal of Medicine*, "lawmakers could lower the price of prescription drugs by reforming the federal 340B Drug Pricing Program. [...]The scope of the 340B program is currently so vast for drugs that are commonly infused or injected into patients by physicians that their prices are probably driven up for all consumers."

Solution: We need to protect the health care safety net by ensuring the underlying market works. The 340B program needs reform and better oversight so that it can benefit patients. Stronger rules for hospitals participating in the program will help ensure the program targets the patients and true safety-net facilities it was intended to help. Specific reforms for hospitals participating in the program should include stricter 340B eligibility criteria; limits on contract pharmacy arrangements; requirements that patients see a benefit from the program; a tighter "patient" definition for eligibility; and limits on which hospital-owned physician practices can participate in 340B.



SOLUTION
#2

Include Prescription Drug Costs In Commercial Insurance Risk Adjuster

Today, U.S. consumers have a wide range of health insurance options. Despite efforts to make sure everyone has health coverage, consumers still have the choice to forgo insurance. As a result, commercial insurers face the risk of adverse selection in which individuals delay getting covered until they know they need services. This risk can raise premiums for everyone.

To mitigate the risk of adverse selection the individual and small group markets employ a range of strategies to promote fairness and encourage enrollment. Insurers are incentivized to price plans at the lowest possible premium, which encourages enrollment but may raise other costs for patients. Other strategies include penalties for not enrolling and a financing mechanism (known as risk adjustment) designed to protect against adverse selection by spreading the costs of the sickest patients across insurers.

The risk-adjustment mechanism for exchange plans currently is hampered by limited data that do not consider prescription drug claims. If risk adjustment is inadequate, there is a danger that insurers have an incentive to discourage enrollment by the sickest individuals. This is in conflict with the government's interest in expanding coverage and with patient interests in gaining access to coverage and care. In other words, the interests of patients, insurers and government would not be well aligned.

Example: Adverse tiering. Adverse tiering is when a health plan subjects all medicines for treating certain conditions to very high cost sharing. The growing prevalence of this practice suggests issuers may face difficulty managing financial risk caused by enrolling too many patients with certain health conditions. The consequence is these patients – often with chronic but manageable conditions – are left with fewer choices or inferior coverage.

Solution: An improved risk adjuster that includes prescription drug data, combined with stronger oversight, could mitigate problems like adverse tiering. The current risk-adjustment model relies on clinical diagnoses and demographic data to predict the relative cost of covering individual or small group policyholders. It does not draw from pharmacy data even though research shows that including the data could make predictions more accurate. Pharmacy data could also help account for costs likely to be incurred by patients who are not enrolled for the whole year or other individuals with chronic conditions managed with medicines rather than frequent medical visits. With a more accurate risk adjuster, incentives would be better aligned across patients, insurers and government, which would help improve affordable access to care for patients.