

American Society of Clinical Oncology Position Statement On Addressing the Affordability of Cancer Drug

Approved by the ASCO Board of Directors June 1, 2017

Introduction

The issue of drug price, particularly in the area of specialty pharmaceuticals, has emerged as a bipartisan concern with both Members of Congress and the Administration. Specialty medications typically include biological products that are often administered by injection or infusion, sometimes require special handling and administration, and are often substantially more expensive than the traditional small molecule drugs.¹ Specialty medications accounted for 37% of drug spending in 2015, and projections are that they will account for 50% of all drug spending by 2018.² Oncology drug pricing is expected to increase at a rate of more than 20% per year for the next several years.³ Healthcare expenditures—including drug costs—have become a major cause of personal bankruptcy, and “financial toxicity” has become a common term used to describe the financial distress that now often accompanies cancer treatment for patients.

Many policymakers consider this a uniquely American problem, as the U.S. healthcare marketplace has few tools to control cost effectively. Projections are that the United States will continue to have the largest per capita drug spending increase of any developed country in the world, while countries like Spain and France will experience per capita spending decreases.⁴

At the same time, the last decade has seen tremendous progress in development of new classes of drugs that have greatly improved outcomes for patients with certain cancers. Immune checkpoint inhibitors, for example, have improved the prognosis for many patients with once rapidly fatal cancers. The speed with which new therapies enter the U.S. drug market and become available to patients tends to be faster than in other countries.⁵ Nevertheless, one recent study revealed that only 19% of recently approved cancer drugs met ASCO’s goals for producing clinically meaningful survival outcomes for patients, despite often entering the

¹ Gleason PP, et al. Health Plan Utilization and costs of specialty drugs within 4 chronic conditions. *J Manag Care Pharm.* 2013 Sep;19(7):542-8: <http://www.jmcp.org/doi/pdf/10.18553/jmcp.2013.19.7.542>

² The Express Scripts Lab. Express Scripts 2015 Drug Trend Report Executive Summary. March 2016. <https://lab.expressscripts.com/lab/drug-trend-report>

³ Ibid

⁴ IMS Institute for Health Informatics. Global Outlook for Medicines Through 2018. <http://www.imshealth.com/en/thoughtleadership/quintilesims-institute/reports/global-outlook-for-medicines-through-2018>

⁵ Kanavos, P. et al. "Higher US Branded Drug Higher US Branded Drug Prices And Spending Compared To Other Countries May Stem Partly From Quick Uptake Of New Drugs", *Health Affairs*, Vol. 32, pp. 753-761, 2013

marketplace at extraordinarily high prices.⁶ Balancing the need for continued innovation for our patients, equitable access to high quality care and unsustainable cost trends calls for bold but thoughtful action.

As the leading professional organization for physicians and oncology professionals caring for people with cancer, ASCO is deeply concerned about the effect rising drug prices have on individuals affected by cancer. We are a patient-centered professional society whose members deliver some of the most complex and expensive treatment regimens in health care during one of the most stressful healthcare episodes in most people's lives. Our members are expert in the technical benefits and risks of these drug regimens and treatment programs but we also witness the financial impact of cancer treatment on patients and families.

ASCO is committed to supporting and promoting practical policy solutions that ensure patients with cancer have access to—and can afford—drugs vital to the treatment of their disease. We propose a number of modest "experiments" to determine if any model can help rein in drug costs without jeopardizing innovation or access to care. We join our colleagues from the American College of Physicians,⁷ the American Academy of Dermatology,⁸ the Council of Medical Specialty Societies⁹—of which ASCO is a member—and the Society of Gynecologic Oncology,¹⁰ who have all recently released positions on high drug prices and spending.

ASCO is firm in its position that any policy solutions to address the price of cancer drugs must promote access to care for patients, affordability of drugs vital to their treatment, and innovation in drug development. Regardless of the specific policy recommendations pursued, defining value must underpin the drug pricing debate. The principles below guided development of ASCO's position:

- Value-based solutions that are patient-centered and evidence-driven should inform drug prices in the United States.
- Oncology professionals should define optimal care and provide a framework to assess the comparative value of cancer treatment options from a clinical perspective.
- There should be a real and consistent relationship between the value of a given drug and its cost to patients.

⁶ Kumar H, Fojo T, Mailankody S. An Appraisal of Clinically Meaningful Outcomes Guidelines for Oncology Clinical Trials. *JAMA Oncol.* 2016;2(9):1238-1240

⁷ Daniel H, for the Health and Public Policy Committee of the American College of Physicians. Stemming the Escalating Cost of Prescription Drugs: A Position Paper of the American College of Physicians. *Ann Intern Med.* 2016;165:50-52

⁸ Position Statement on Patient Access to Affordable Treatments, American Academy of Dermatology Association, 2015. <https://www.aad.org/Forms/Policies/Uploads/PS/PS%20-%20Patient%20Access%20to%20Affordable%20Treatments.pdf>

⁹ Council of Medical Specialty Societies. CMSS Principles for Increasing Access to Needed Medications by Patients, 2016. <https://cmss.org/cmss-principles-for-increasing-access-to-needed-medications-by-patients/>

¹⁰ Society for Gynecologic Oncology. Addressing The High Cost of Drugs for Oncology Patients. <https://www.sgo.org/publicpolicy/addressing-the-high-cost-of-drugs-for-oncology-patients/>

- Physicians do not control the launch price of drugs. However, physicians do determine how drugs are used and are accountable for appropriate utilization.
- Cost-containment strategies should not limit the ability for patients to receive access to appropriate care, or for providers to prescribe such care.
- Cost-containment strategies should incentivize—not hamper—innovation that results in clinically meaningful improvements in patient outcomes.

Within this statement, we review a number of solutions that policymakers have proposed as a means of addressing the soaring prices of specialty drugs. We provide ASCO’s perspective on whether these proposals should be tested, primarily from the standpoint of impact on patient care. We use both the term “drug pricing” and “drug spending” throughout this statement. We refer to “drug pricing” as the unit cost of the drug; “drug spending” represents the combination of price and utilization.

Defining Value in Cancer Therapeutics

ASCO has launched a number of programs designed to address the rising cost of cancer care in general, beginning with a 2009 ASCO Guidance Statement on the Cost of Cancer Care, continuing with efforts that include participation in the Choosing Wisely campaign and, most recently, the publication of ASCO’s Value Framework.¹¹¹² The Value Framework helps oncologists and patients assess treatment options by providing a standard measure of net health benefit. ASCO also has worked to address the cost and quality of cancer care—apart from drug price—through initiatives such as its quality improvement program, the Quality Oncology Practice Initiative (QOPI®); encouraging use of high value clinical pathways; setting the bar for clinically meaningful outcomes in cancer clinical trials; and advancing payment reform through the Patient-Centered Oncology Payment Model (PCOP). These efforts have focused on cost reduction by encouraging appropriate resource utilization, with the goal of reducing excess spending associated with unnecessary or inappropriate care.

We are not alone in these efforts. The European Society for Medical Oncology (ESMO) has released a value framework very much compatible with the ASCO framework. Other serious efforts to describe value include the Memorial Sloan Kettering Drug Abacus, the Institute for Clinical and Economic Review (ICER) collaborative evaluation model, and the NCCN evidence block initiative. We are encouraged by these mature efforts, which demonstrate that a group of engaged stakeholders can provide the expertise to define and assess the value of cancer therapies. However, establishing a patient-centric, robust and broadly applicable value framework requires the assessment of a broader range of clinical trial endpoints during drug research and development. In particular, it requires collection of validated quality of life and patient-reported outcome measures for drug registration trials. It also requires rapid expansion

¹¹ Schnipper LE, Davidson NE, Wollins DS et al. Updating the American Society of Clinical Oncology Value Framework: Revisions and Reflections in Response to Comments Received. *J Clin Oncol* 34(24): 2925-2934, 2016

¹² Meropol NJ, Schrag D, Smith TJ, et al. American Society of Clinical Oncology Guidance Statement: The Cost of Cancer Care. *J Clin Oncol* 27(23): 3868-3874, 2009

of big data projects such as ASCO's CancerLinQ™ that collect real world outcomes that allow comparison of drug safety and effectiveness outside the setting of formal clinical trials.

For all stakeholders, the definition of value ultimately comes down to the relationship between price and meaningful improvements in health outcomes at the level of individual patients—and society more broadly. Optimizing the value of a new product begins with innovation to address an unmet medical need, followed by clinically meaningful improvements in health outcomes through well-designed and efficiently conducted clinical trials. Effectiveness research is essential to determine how well the new product performs compared to available alternatives—and its impact on more diverse populations than those typically included in the clinical trials used to establish efficacy. Patient goals, preferences, and choices shape the real world experience with a new product, and the direct and indirect costs of treatment to both patients and their families affect its widespread adoption. The medical community should be judicious in using new and costly products until there is clearly established value and clear understanding of how that value relates to treatment goals, available options, and the unique needs, preferences, and goals of individual patients. Doctors should also make sure their patients are aware of the cost, benefit, and personal financial impact of their treatment options and choices.

Research in many domains is necessary to improve assessment of the value of new cancer treatments. Advancing our understanding of value requires development of new clinical efficacy endpoints, both provider and patient-reported, that accurately describe how a patient functions and feels. These endpoints should reflect outcomes of value to patients other than survival, particularly in non-curative settings. Better predictive biomarkers can transform a drug of modest efficacy in an unselected population to one of high efficacy in a biomarker-defined subgroup, and thereby contribute to improving the value of a given treatment.

Policy initiatives that affect market approval, reimbursement, or price all deserve careful consideration to determine how well they balance cost while preserving both innovation and patient access to life-improving therapies. In what follows, ASCO proposes consideration of strategies that could be pilot-tested with a goal of improving the value of cancer care.

Ensuring High-Value Drug Development

In 2014, ASCO's Cancer Research Committee published a statement, "Raising the Bar for Clinical Trials by Defining Clinically Meaningful Outcomes."¹³ The committee focused on several cancer scenarios in the metastatic setting, with a primary focus on median overall survival and hazard ratios. Secondary endpoints were improvement in 1-year survival rates and progression free survival. Using front line metastatic pancreatic cancer as an example, the statement suggested that any new therapy should demonstrate a median survival improvement of 4-5 months (HR 0.67-0.69) and a minimum 1-year survival improvement from 48% to 63% in order to meet the

¹³ Ellis LM, Bernstein DS, Voest EE, et al: American Society of Clinical Oncology perspective: Raising the bar for clinical trials by defining clinically meaningful outcomes. *J Clin Oncol.* 2014 Apr 20;32 (12): 1277-80

definition of “clinically meaningful.” The goal of these recommendations was to encourage clinical trial developers to set higher goals for improving patient outcomes. As such, the recommendations also serve to provide an important context for the assessment of a new cancer treatment. To ensure the development of high-value drugs in cancer care, the Food and Drug Administration could limit its approval for indications/therapies to those that meet or exceed these recommended incremental benefits, rather than focusing on small benefits that achieve statistical significance in large trials.

Testing Different Value-Based Pricing Strategies

Value-based pathways are an approach that could be used to better align the pricing and utilization of drugs with the value they bring to patients. To test this approach, appropriate drug utilization would be used as a quality measure instead of a resource-use metric; drug therapies would be placed in hierarchical pathways based on their comparative value; and practice performance scores would be based on appropriate use of pathway recommendations. Practices that fall below a certain threshold would receive a negative adjustment in payment. This has the advantage of incentivizing both provider use of higher value treatments and development of therapies by the pharmaceutical industry that achieve high value through a combination of maximizing efficacy and minimizing toxicity and costs.

Another approach worthy of consideration is indication-specific pricing. Under such an approach, payment for the same drug would vary depending on its effectiveness in different approved indications. This approach requires the ability to compare relative value, again emphasizing the need for a widely accepted mechanism to determine value.

Outcomes-based pricing is another frequently discussed approach to controlling cost and improving value. In this scenario, reimbursement depends on how well the drug works in a particular patient. For example, if a patient survives beyond the median survival reported in the clinical trial population, reimbursement is higher than a stated benchmark. Conversely, if the drug therapy results in less than the expected median survival time, reimbursement would be lower. Payers could deploy this approach at the population level, i.e., if a drug does not perform in the actual treatment population as indicated by the trial data, manufacturers would provide discounts/rebates to payers/patients. This approach requires agreement on average or baseline price and that would best be determined using a value model as above.

An approach that ASCO does not support is the use of *payment bundles* to control drug costs. Under such an approach, all costs for treating a patient, including drugs, are bundled into a single episode-based payment. Payment bundles do not affect price directly. Further, bundled payment programs create circumstances that could force providers to make suboptimal or lower value choices. While appealing in the abstract to many in the health policy world, such bundles will likely never be sensitive enough in a world of increasing precision-based therapy and heterogeneous patient populations to account for appropriate variation in drug prescribing. ASCO is firm in its belief that no provider should experience financial penalty for providing the right drug to the right patient at the right time.

Encouraging Development and Use of Generics and Biosimilars

ASCO strongly endorses the position expressed by the American College of Physicians in opposition to “extending market or data exclusivity periods beyond the current exclusivities granted to small-molecule, generic, orphan, and biologic drugs.” We further agree that the provision in President Obama’s 2016 budget to reduce data exclusivity on biologics from 12 to 7 years is worthy of 6 consideration. We additionally agree with several other provider organizations that practices such as product hopping, evergreening, and pay for delay should not be allowed. According to the Federal Trade Commission (FTC), the tactic known as “product hopping” or “product switching” occurs when brand name pharmaceutical companies introduce reformulations of their branded product that offer little or no therapeutic advantage.¹⁴ Similarly, “evergreening” occurs when brand name companies patent as new drugs slight modifications of old drugs.¹⁵ This allows drug companies to maintain market share after drug patents expire. The company can withdraw its branded product, forcing patients to use its reformulated version, thereby obstructing generic competition and enabling the company to keep its market exclusivity. “Pay for delay” is a legal tactic in which branded drug manufacturers slow or obstruct generic competition by paying companies not to introduce lower cost alternatives to the marketplace. The FTC has estimated this practice costs consumers and taxpayers \$3.5 billion in higher drug costs each year.¹⁶ By definition, these strategies represent higher cost without meaningful improvements in care, a result that is not in the best interest of patients.

Limiting the Financial Burden that Payer Policies Place on Patients

While ASCO shares the overall goal of supporting value-based care, certain cost containment approaches used by a growing number of payers threaten to undermine patient access to medically necessary oncology care. In particular, ASCO strongly opposes the trend toward tiered formularies. This approach places specialty drugs in the highest tiers, which carry higher percentages of coinsurance. This places vulnerable patients in the cross hairs of a problem they did not create. If their disease requires the use of an effective and high value therapy, they should not be asked to bear the financial burden of the higher price tag associated with this necessary—and sometimes life-saving— treatment. As with our objection to the bundling of drugs stated above, shifting this problem to patients who are receiving the right drug at the right time is not an acceptable solution.

¹⁴ FTC Files Amicus Brief Explaining That Pharmaceutical “Product Hopping” Can Be the Basis for an Antitrust Lawsuit. November 2012. <https://www.ftc.gov/news-events/press-releases/2012/11/ftc-files-amicus-brief-explaining-pharmaceutical-product-hopping>

¹⁵ Krans B. Pharmaceutical ‘Evergreening’ Raises Drug Costs, Study Says. Healthline News June 2013. <http://www.healthline.com/health-news/policy-drug-companies-use-evergreening-to-extend-market-share-060413>

¹⁶ Federal Trade Commission. Pay-for-Delay: How Drug Company Pay-Offs Cost Consumers Billions. An FTC Staff Study January 2010. <https://www.ftc.gov/sites/default/files/documents/reports/pay-delay-how-drug-company-pay-offs-cost-consumers-billions-federal-trade-commission-staff-study/100112payfordelayrpt.pdf>

Medicare Negotiation of Drug Rebates

Current law prohibits the Medicare program from negotiating volume discounts with manufacturers. Significant savings may be possible through such an approach, exemplified by the fact that private Part D pharmacy benefit managers do, in fact, negotiate with manufacturers for rebates and achieved rebates totaling \$6.5 billion in 2008.¹⁷ While there is no question Medicare could use its market power to extract discounts and rebates as is done by Medicaid and the Veterans Administration system, there are several cautions to this approach. First, doing so effectively would ultimately require that Medicare have the ability to deny coverage of an FDA- approved drug if it deems the price to be above an assessed value. Whether the United States is willing to give Medicare such power requires considerable thought and debate. Second, at least a portion of the cost savings obtained by Medicare is likely to be shifted to private payers who have less negotiating power, which limits the societal impact of this approach. An alternative strategy would be for Medicare to require the use of valuebased pathways as outlined above. In this way, the community at large—not the government— establish value. We recommend that Medicare test a value-based pathway approach to reimbursement to determine its feasibility.

Transparency of Drug Costs

All provider organizations that have issued statements on drug pricing have endorsed greater transparency on drug pricing. Doing so requires that manufacturers disclose material and production costs, research and development costs (including those for drugs acquired from other companies), marketing costs, and any federal research dollars that contributed to the discovery, research and development of the drug. Such transparency would allow payers and patients to at least make some informed comparison of the relationship between development costs and price for drug products and exert public pressure on companies where the two appear to be widely divergent. Although ASCO supports the general premise of testing price transparency as a means for consumer and provider education, we note that a validated, agreed-upon methodology for value-based pricing could accomplish the same goal.

Re-importation of Drugs

This strategy assumes that all other developed countries in the world have some regulatory framework in place to control the quality and price of drugs. It also assumes that re-importation of these lower priced drugs would have a downward pressure on prices charged in the United States. Testing this approach would require consideration and resolution of a number of safety concerns. In addition, given the dynamic nature of world markets, widespread use of this

¹⁷ Daniel H for the Health and Public Policy Committee of the American College of Physicians. Stemming the Escalating Cost of Prescription Drugs: A Position Paper of the American College of Physicians. Ann Intern Med. 2016

practice would almost certainly cause the price of drugs to rise in other countries, mitigating some, if not most, of the cost savings.

Conclusions & Recommendations

Rapidly rising drug prices and spending in the United States have engendered considerable passion and debate among all stakeholders in the system about how to constrain costs. Some proposals target market dynamics to control price, while others target provider and patient utilization to control spending. There is a growing call for more transparency by drug manufacturers, with particular emphasis on drugs that received federal funding or philanthropic support at any point during their development. There is also discussion about increasing Medicare's ability to leverage its market power in order to negotiate better drug prices for its beneficiaries (although there are few specifics on how this might work, or evaluation of potential unintended consequences).

Some of these strategies are worth exploring, but the ultimate solution to improving the affordability of drugs requires accelerated efforts to define value. The notion of value-based systems in health care has moved beyond a theoretical concept put forward by academics. Rather, it has been the subject of tangible, published efforts using real patient data in the United States and Europe. With appropriate authorization by Congress to identify a standardized, value-based evaluation of therapeutics, the community at large could deliver a model in short course. Moreover, with a standard framework for defining and assessing value, testing multiple value-based pricing models is possible. A valid and reliable framework, one that is evidence-based and patient-centered, could support value-based approval of new therapies.

Recommendations

Recognizing that many are actively engaged in this issue, ASCO makes the following recommendations as guidance to any ongoing and future efforts to address the affordability of cancer drugs in the United States either by the Administration, Congress, or other entities.

- *Solutions to address the affordability of cancer drugs—many of which are highlighted in this statement—should be identified, evaluated, prioritized and tested.*
Any of the approaches examined earlier in this statement may lead to an array of unknown impacts. Efforts to address the affordability of cancer drugs must recognize the potential of unintended consequences and, therefore, should be carefully tested in pilot projects before a widescale, national launch.
- *The larger community—including providers, patient advocates, payers, hospitals, experts in health economics and health outcomes, representatives from the pharmaceutical and biotechnology industries, Members of Congress, and Administration policy makers—must actively participate in any effort to develop policy solutions to address the affordability of cancer drugs.*

There is no simple solution to escalating drug prices, and many differing views on what constitutes value in cancer treatment. ASCO believes that active dialogue and engagement by all interested parties must be a centerpiece of efforts to address this issue—particularly with the involvement of patients, who will be directly impacted by proposed solutions, and physicians, who have the expertise to define clinically sound care.

- *Congress and/or the Administration can play an important role in bringing together a diverse group of experts to identify, evaluate, and prioritize a series of demonstrations designed to test some of the solutions highlighted in this statement—and, once tested, to recommend implementation for those that are successful. A high-priority effort of this group should be to propose a strategy for blending various value frameworks into a transparent and standardized approach to assessing value, and recommending drug pricing and reimbursement based on the value delivered.*

As noted earlier, many private initiatives have developed tools to assess the value of cancer drugs. ASCO recommends that efforts be advanced to articulate a universally accepted definition of value in cancer care and to evaluate existing value frameworks for synergistic opportunities and possibly combine them into a single approach for use by physicians with their patients, policymakers, payers, manufacturers, and others.

Solutions to rising drug prices and spending should be considered with the following driving principles in mind:

- Patients should have access to life- prolonging and improving treatments and should not suffer financial harm when receiving the care they need.
- Providers should be confident they have support in delivering the right treatment at the right time to the right patients.
- Manufacturers and the investment community should continue to see value in high-risk, high-reward science.

We must balance these principles with recognition of the financial toll of drug costs on private and public budgets. ASCO contends that solutions centered on value stand the best chance of achieving this balance in the most equitable and effective way. Drawing on the collective knowledge of its more than 40,000 members, ASCO stands ready to work together with the larger community to define, test and agree upon solutions to ensure access, affordability and innovation—with the ultimate goal of ensuring the health and well-being of the patients our members serve.

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