

MEETING SUMMARY
PRESIDENT'S CANCER PANEL
PRICING AND PAYMENT STRATEGIES FOR CANCER DRUGS:
MAXIMIZING PATIENTS' ACCESS TO BENEFICIAL THERAPIES

March 27, 2017
Philadelphia, PA

This workshop was the third in the President's Cancer Panel's (the Panel) 2016–2017 series on access to and cost of cancer drugs. The workshop brought together experts in drug pricing and payment from the biopharmaceutical industry, insurance companies, healthcare organizations, and professional oncology organizations, along with patients and patient advocates, health economists, and other academic researchers. Participants were encouraged to live-tweet at [#CancerRxValue](#) during the workshop. This meeting summary was prepared to satisfy requirements established by the Federal Advisory Committee Act. The summary provides an overview of presentations and discussions occurring during the workshop and does not necessarily reflect the views of Panel members.

President's Cancer Panel

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WELCOME AND INTRODUCTIONS

DR. BARBARA RIMER

Dr. Rimer welcomed invited participants and other attendees to the meeting on behalf of the Panel. She introduced Panel members, provided a brief overview of the history and purpose of the Panel, and described the aims of the current series of meetings. Dr. Rimer thanked Dr. Ann Geiger and acknowledged Dr. Gary Gilliland (who was unable to attend the workshop) for their assistance in planning the series. She also introduced workshop facilitator Robert Mittman and Panel staff members.

Pharmaceutical innovation has created potential for meaningful, life-prolonging gains and, in some cases, cures for cancer patients. However, there is growing concern that the high costs of cancer drugs may hinder access for cancer patients, many of whom experience financial toxicity as a result of their cancer care. It is important to identify solutions to ensure drug affordability and access without sacrificing quality or innovation.

OPENING ROUNDTABLE

The objectives of the workshop were to (1) review key factors that affect pricing of and payment for cancer drugs, (2) outline alternative approaches to cancer drug pricing and payment, (3) identify barriers to implementing alternative approaches to cancer drug pricing and payment, and (4) identify potential actions to overcome barriers. During the opening roundtable, each participant was asked to identify a single change in cancer drug pricing and payment that would increase patients' access to high-value cancer drugs. Suggestions included:

- Develop a definition of value accepted by all stakeholders.
- Improve market competition to drive down prices of cancer drugs.
- Gather real-world evidence to provide insight into the value of drugs, including for different patient populations.
- Create policies that promote timely introduction of generic drugs to the market.
- Develop non-drug-based therapies that will enhance or provide an alternative to drugs.
- Create safe harbors to allow pharmaceutical companies and payers to develop creative contracting strategies.
- Eliminate coverage mandates for cancer drugs.
- Allow payers to refuse coverage of low-value drugs.
- Develop ways to link drug price to value and/or individual patient outcomes.

- Improve physician-patient communication about goals and costs of cancer care to promote informed decision making.
- Encourage patients and physicians to choose high-value drugs and avoid low-value drugs.
- Eliminate physician incentives to recommend treatments based on factors other than clinical value.
- Decrease out-of-pocket expenses for cancer patients, particularly those in vulnerable financial situations. In particular, benefit plans should be designed so that patient costs are based on the net price of drugs rather than the list price.
- Increase the number of patients participating in clinical trials.

PRICING AND PAYMENT LANDSCAPES—FRAMING THE VALUE OF CANCER DRUGS

THE PRICING AND PAYMENT LANDSCAPE—FRAMING THE VALUE OF CANCER DRUGS

Clifford Hudis, MD, FACP, Chief Executive Officer, American Society of Clinical Oncology (ASCO)

Key Points

- A quote commonly attributed to Warren Buffet is relevant to cancer drug pricing: “Price is what you pay. Value is what you get.”
- Traditional economic rules do not apply to cancer drug pricing because (1) the buyer is not the end user and beneficiary and (2) the values of the buyer and user may not align. Additionally, there is little transparency regarding drug efficacy and toxicity, which makes it difficult to compare available options.
- The United States does not have a single healthcare system with shared values and a common goal. Various stakeholders have different goals, which often are in conflict.
- Patients are somewhat insulated from the overall cost of a drug, but they are affected by out-of-pocket costs. When out-of-pocket costs for a drug are high, some patients take less than the prescribed amount, partially fill their prescriptions, or avoid filling their prescriptions altogether. If a truly transformative drug is available, it would make more sense for a third-party payer to create a financial incentive for patients to take the drug than force patients to pay.
- ASCO has a longstanding commitment to high-quality, high-value cancer care. In 2009, ASCO’s Cost of Cancer Care Task Force issued guidance on this topic, and ASCO began providing toolkits to help patients and families manage the costs of cancer care. ASCO has participated in multiple rounds of the Choosing Wisely initiative. It also has developed a patient-centered oncology payment reform model intended to help control costs as the Medicare Access and CHIP Reauthorization Act is implemented.
- It is challenging for ASCO and oncologists to engage in discussions about the costs of cancer care because many view this as a political issue. Some people have questioned whether ASCO is advocating in the best interests of patients, and there are also some who think “value” is code for rationing. However, ASCO surveys have found that at least some patients want to discuss cost with their doctors.
- ASCO has developed a value framework intended to facilitate conversations about value and enable informed, shared decision making by doctors and patients. The three primary parameters included in the framework are clinical benefit, toxicity, and cost. The goal is to create a tool that can be customized for each patient (e.g., importance of side effects versus clinical benefit). Guiding principles of the framework include: (1) the central importance of the physician-patient relationship in defining management options for the patient, (2) the need for patient access to both clinical and cost

information to ensure informed decision making, and (3) the responsibility of physicians to be good stewards of healthcare resources in addition to serving as advocates for their patients.

- ASCO published a conceptual framework for assessing the value of new treatment options compared with existing options in a 2015 *Journal of Clinical Oncology* [article](#). Different methodologies are presented for advanced disease and adjuvant settings. Input was solicited from numerous stakeholders including advocates, oncologists, insurers, pharmaceutical companies, regulators, and others.
- The framework provides standardized information to doctors and patients, but it is not a ranking system that allows comparison of any two drugs. A key output of the framework is net health benefit, which represents the added benefit patients may receive from a new cancer drug compared with standard of care. When calculating clinical benefit, effects on overall survival are weighted most heavily, followed by progression-free survival and response rate. Bonus points are awarded for improvements in quality of life, and penalties are imposed for toxicities. The net health benefit score is presented alongside cost information.
- ASCO solicited feedback on its conceptual value framework and received more than 400 comments from the community. A revised version of the framework was published in a 2016 *Journal of Clinical Oncology* [article](#).
- ASCO is working to further improve the value framework methodology. For example, efforts are ongoing to determine how to do meaningful cross-trial comparisons. Patient engagement also will continue in order to identify key endpoints most meaningful to patients and develop validated assessment measures so these endpoints can be included in the framework.
- While ASCO hopes that the value framework will be a useful clinical tool, a third-party mandate that it be used is not advisable. It is important that doctors are supported so they can be empathetic caregivers, but adding to the long list of requirements is not the way to accomplish this.

CANCER DRUG PRICING

Caroline F. Pearson, Senior Vice President, Policy and Strategy, Avalere Health

Key Points

- There are three versions of price for a given drug: (1) list price, which is the price set by the manufacturer, (2) negotiated price, which is the net price paid by the payer, health plan, or pharmacy benefit manager, and (3) the price paid by the patient.
- Manufacturers consider a number of factors when setting the list price for a drug, including clinical value of the drug relative to other drugs, prices of other products on the market, expected rebates, and expected contributions to patient assistance programs. Drugs entering the market also must bring in enough revenue to cover research and development for both successful and failed drugs.
- The difference between list price and negotiated price can be significant. This difference depends largely on the negotiating power of the payer. Markets for different diseases are very different. The market for some diseases (e.g., diabetes) is very competitive, but cancer drugs are generally more differentiated so there is less head-to-head competition.
- Patient out-of-pocket costs for healthcare, including drugs, have risen dramatically in recent years. Most patients' insurance policies require them to pay coinsurance for specialty drugs, including cancer drugs; in 2016, the average coinsurance for specialty drugs was 26 percent. Patients are upset about how much they have to pay for drugs.
- It is difficult to define and measure value, particularly outside the context of randomized clinical trials. Value should be considered in terms of the total cost of cancer care, not only the cost for drugs. In some cases, drugs may help reduce overall costs of cancer care.

- Avalere has been working with FasterCures to incorporate the patient perspective into emerging value frameworks. Patients are concerned about issues other than the cost of a drug, including mode of administration and treatment schedule. Patients also value different things: one patient may want to extend life while another would rather maximize quality of life.

PAYER PERSPECTIVE ON CANCER DRUG PRICING

Lee Newcomer, MD, MHA, Senior Vice President, Oncology, UnitedHealth Group

Key Points

- The various parties involved in cancer drug pricing act in response to the incentives that are embedded in the current system. If change is desired, incentives must be altered.
- Classic free-market principles do not apply to drug pricing in the United States. This is in part because 42 states and federal programs require insurance companies to cover all U.S. Food and Drug Administration (FDA)-approved cancer drugs, which eliminates the ability of payers to negotiate prices with drug manufacturers.
- Introduction of competitor drugs to the marketplace is expected to exert downward pressure on price, but this does not happen for cancer drugs. When Gleevec was approved about 15 years ago, it was priced at \$35,000 per year. Though expensive, it was viewed as a good value because it cured people. When a competitor drug came to market a few years later, it was priced at more than \$110,000 per year, and the price of Gleevec subsequently increased to a similar level. Payers are required to cover both drugs.
- A new drug for non-small-cell lung cancer provides negligible improvement in progression-free survival and causes cardiac arrest in 3 percent of patients. Despite minor impact on disease progression, patients who received the drug lived about 6 weeks longer than those treated with standard therapy. The new drug is priced at \$10,000 to \$11,000 per month, compared with \$1,000 per month for standard therapy. Payers are required to cover both drugs.
- Cancer drug coverage mandates were implemented in the 1970s and 1980s because of concerns that payers would not cover new drugs considered at that time to be expensive. Though well intended, these laws have contributed to high prices for cancer drugs and should be eliminated.
- Transparency regarding the clinical risks and benefits of cancer drugs is needed. UnitedHealth is tracking cohorts of patients being treated with various National Comprehensive Cancer Network (NCCN)-recommended regimens. Claims data (e.g., duration of treatment, hospitalizations) are being used to monitor patient outcomes and total costs of care. For example, for one non-small-cell lung cancer regimen, average duration of first-line therapy is 61 days, far shorter than the 6- to 9-month duration of treatment for participants on FDA registration trials. Hospitalization rates range from 1 in 4 to 1 in 3 patients, and total costs of care are between \$1,000 and \$2,000 per day.
- UnitedHealth intends to share the results of its analysis with physicians, patients, and the public by the end of the year. It is hoped that these data will spur conversations between physicians and patients about the value of various treatment regimens.
- Clinical trials need to be more widely utilized in adult oncology.
- Insurance companies pay a different markup on drug prices depending on where a drug is administered. Cancer drugs administered in hospitals have an average markup of 148 percent, compared with 28 percent for those administered in physician-owned practices.
- The average U.S. family of four has an annual household income of \$52,000 and spends \$27,000 per year on health insurance and out-of-pocket expenses for healthcare. The increasing costs of healthcare are unsustainable. Something must be done to reverse this trend.

DISCUSSION

Defining and Incentivizing Value

- Value depends on frame of reference—it will be defined differently for patients compared with payers. Factors that influence drug value were discussed. Clinical benefit contributes positively to value, while toxicities and cost contribute negatively. Value calculations also should take into account type of cancer and whether the cost of a drug is offset by reductions in other costs (e.g., hospitalization). Hope also plays a role in patients' value assessment, particularly if there is no clear evidence that a drug is ineffective.
- The word “value” has different connotations for different people. Physicians and academics may think of value as the sum of a drug’s benefits and risks, but many people in the general population equate the word “value” with “cheap.”
- When considering drug value, it is important to think about the effectiveness of cancer drugs and the physical toll they often take on patients. In many cases, patients suffer serious side effects to gain only a few months of life. This level of effectiveness would not be acceptable in other disease areas.
- PhRMA, along with several other stakeholders, participated in the Turning the Tide Against Cancer initiative. As part of this initiative, there was a recommendation to develop a consensus definition of value. There was agreement about value at a conceptual level, but there was recognition that determining value in healthcare, and in oncology in particular, is complicated. Challenges include identifying ways to measure value, defining patient populations, and establishing timeframes for value measurement, among other things.
- The best way to ensure value is to incentivize development of innovative drugs with potential to transform patient outcomes—to turn a fatal disease into a curable disease. This requires investment in scientific research. Drugs that extend life by weeks or a few months do not provide high value to patients.
- Consideration should be given to whether current financing and payment structures incentivize innovation. Overpayment for low-value drugs creates disincentives for innovation. Current venture capitalist approaches focus on the potential profitability of ideas, but there may be value in ideas that do not have high promise of profit.
- The population-level tradeoffs of high drug prices should be considered. Dollars spent on cancer drugs are dollars that cannot be spent on other things, including other health problems or wages.
- Bevacizumab received accelerated approval from FDA for treatment of breast cancer, but this indication was removed about two years later when confirmatory trials failed to show a benefit for breast cancer. Dr. Hudis recounted that patients he was treating for breast cancer were upset the drug would no longer be covered, but not a single patient with the means to pay for the drug out of pocket chose to do so. They did not view the drug as high value when they were required to pay for it themselves. This illustrates how the payment structure for cancer care makes it difficult to assess the individual patient’s sense of value.
- Gleevec is an example of a drug that provides high value to patients. Early data indicated that patients on Gleevec stayed out of the hospital, did not require bone marrow transplants or splenectomies, and generally felt better than patients not on the drug. The drug was approved based on short-term clinical response data, and it was later shown to lead to long-term remission for about 90 percent of patients.

Payment and Reimbursement

- Drug pricing and negotiation dynamics are different for physician-administered drugs covered by Medicare Part B and oral drugs covered by Medicare Part D. In general, the market for drugs covered through a pharmacy benefit (e.g., Medicare Part B) is more competitive than for physician-

administered drugs, although many of the negotiation strategies (e.g., formularies, rebates) used for other types of drugs are not effective for cancer drugs because of coverage mandates.

- Dr. Newcomer reported that, for cancer drugs, payers have more negotiating power for physician-administered drugs in private oncology practices than for drugs administered in hospitals or hospital-owned facilities.
- Dr. Sollano reported that about 50 percent of Medicare Advantage plans are negotiating with Pfizer for some of the new oral cancer drugs. Dr. Newcomer indicated that negotiations do happen, but they are generally not very effective, with payers often receiving only small price concessions for cancer drugs.
- Many drugs are used off-label in oncology. Insurance companies can implement additional rules about how drugs can be used off-label. Dr. Newcomer noted the importance of distinguishing between off-label and off-evidence use of drugs in cancer. There is strong evidence of effectiveness for some off-label indications. Payers will be punished in the marketplace if they do not provide coverage for evidence-based, off-label use. However, insurance companies are more likely to refuse coverage or discourage utilization of drugs off-label when there is no supporting evidence.
- Dr. Newcomer expressed his view that tiering can be an effective utilization management strategy if there are a number of drugs for a given indication, but this often is not the case for cancer. Tiering is a flawed long-term strategy if it discourages utilization of highly effective drugs.
- When Sovaldi was approved for treatment of hepatitis C, it entered the market at a price that was about twice as high as had been expected because the company that developed the drug was acquired by another company. In addition to the higher-than-expected price, there was the huge backlog of patients who needed to be treated. This created a budget surge for which payers were not prepared. Fortunately, competitor drugs entered the market and prices came down. If the same scenario occurred for cancer drugs, prices likely would not come down because of coverage mandates.
- Individuals frequently change health insurance plans. Thus, a payer that covers the cost of a very expensive drug like Sovaldi may not reap the long-term savings. Mr. Barrueta indicated that even for Kaiser Permanente, which experiences less member turnover than other payers, Sovaldi did not result in large-scale savings. This was in part because savings calculations were largely based on avoidance of liver transplantation for patients treated with Sovaldi. Though transplants among hepatitis C patients declined, the overall number of liver transplants did not decline because livers that would have been used for hepatitis C patients were used for other patients instead. Payers cannot assume that long-term savings will cover the cost of expensive drugs.

Other

- Patients need a navigator to help them deal with the healthcare system, including their own physicians. Patients are often not aware of resources available to them and may not feel empowered to question the judgment or decisions of their physicians.
- The 340B Drug Pricing Program has deviated from its original intent and should be reformed. Hospitals participating in the 340B program make tens of millions of dollars because of the discounts they receive on cancer drugs.
- One reason cited by manufacturers for high cancer drug prices is that the population of patients treated with a given cancer drug is relatively small, so higher prices are needed to cover research and development costs.
- The federal government has a legal right to license inventions made with federal funding, but this right has rarely been exercised.

DISCUSSION AND IDENTIFICATION OF POTENTIAL RECOMMENDATIONS

Participants were asked to brainstorm and identify potential recommendations for Panel consideration in three areas: (1) drug pricing strategies, (2) access to treatments, and (3) communication about treatments. Panel members emphasized that the ultimate goal is to ensure patients' access to high-value drugs. Drug cost is a component of value, but other factors are also important. Decreasing the cost of cancer drugs is desirable, but not at the expense of innovation.

DRUG PRICING STRATEGIES

Alignment of Value and Price

- Participants identified several factors that make it difficult to link prices of oncology drugs to value, including: patent protection, the 340B Drug Pricing Program, extension of market exclusivity through regulatory mechanisms or secondary patenting, overestimation of drug benefit because clinical trial participants are unrepresentative of the overall patient population, mandated insurance coverage for cancer drugs, inability of Medicare to negotiate drug prices, inadequate data on quality of life and survival, lack of consensus about the level of clinical improvement that warrants coverage, and direct-to-consumer and direct-to-physician marketing by pharmaceutical companies.
- A set of principles should be developed to guide policy discussions and decisions about drug value. Value assessments must consider the patient voice.
- Mr. Burkholder asserted that the pharmaceutical industry strongly supports the idea of a healthcare payment and delivery system that supports and incentivizes value. However, discussions of value should include all healthcare services and products, not only drugs. Value assessments must be patient centered and account for differences among patients.

Patients' Perspectives

- Patient input should be included throughout the drug development process. Patient-reported and patient-centered outcomes should be included in clinical trials.
- Patients need better information about how potential treatments will make them feel and how much therapies will cost, in addition to expected impact on survival. Patients want to know how their everyday lives will be affected (e.g., whether they will be able to work, whether they will need to travel to the clinic to be treated). With these types of information, patients will be able to identify treatments aligned with their values.
- Patients often overestimate the likelihood that a treatment will cure them of their disease. It is unclear whether patients will choose to forego an expensive treatment even if they are told that only a small percentage of patients exhibit meaningful responses to the drug. Patients often hope that they will be one of the few who benefit. Patients may make different decisions if they know how a drug is likely to affect their quality of life.
- Physicians may or may not be the best people to provide cost information to patients. More research is needed on the best ways to communicate with patients about various aspects of value among treatment options.
- Patients do not want to be forced to try a less effective therapy as a first-line treatment just because it is less expensive.

Efficient Development of High-Value Treatments

- Pharmaceutical companies strive to develop innovative drugs with large therapeutic effects, but many drugs have only incremental benefits. Though incremental benefits are not the goal, they can eventually result in meaningful benefits for patients.

- It would be more efficient if a common control group were used to test multiple drugs of the same class. However, head-to-head comparisons are not desirable for pharmaceutical companies. They are primarily concerned with generating data to support marketing rather than scientific discovery.
- It would be useful to determine the extent to which public funding has contributed to the development of individual products.
- Drugs can be approved by FDA based on very modest improvements in survival. For example, one pancreatic cancer drug extends life by only 12 days. Many drugs also are approved based on surrogate endpoints that may not be strongly linked to survival or improvements in quality of life. One participant cautioned against trying to define a clinical efficacy threshold for FDA approval since some drugs may provide a more favorable treatment schedule or side effect profile. Rather, there should be efforts to ensure that market prices reflect the overall value provided by a drug.
- Companion diagnostics that identify patients who are likely or unlikely to respond to a given drug are needed. Approval and reimbursement processes for companion diagnostics must be improved.
- Potentially effective interventions may not attract investment by venture capitalists if they will not be financially lucrative. Dr. Green reported that she was advised to incorporate tumor-targeting antibodies into her nanoparticle-based experimental cancer therapy, which was highly effective in mouse models, so that it would be more attractive to investors.

Pricing and Reimbursement

- The current Medicaid rebate scheme (i.e., “best price” rule) deters private negotiation of prices. Manufacturers will not want to negotiate low prices, even for special circumstances (e.g., outcomes- or indication-based pricing), if they will be required to match that price for Medicaid.
- Pharmaceutical companies and payers are prohibited by anti-kickback rules from working together to discuss value and pricing.
- Healthcare should be viewed as a natural utility. For other natural utilities, like telephone service, prices are set or the owner of the utility subsidizes people who cannot afford it. However, anti-kickback rules prevent pharmaceutical companies from offering certain discounts to people covered by federal health care programs. Those struggling to pay for their drugs are forced to receive support indirectly through charitable groups, which are notoriously inefficient.
- Outcomes-based pricing should be explored. Initially, it may only be feasible to establish prices based on aggregate outcomes, but it may eventually be possible to determine price based on responses of individual patients.
- Value-based pricing and reimbursement will be facilitated by ongoing collection of data on clinical outcomes and entry of generics and biosimilars into the marketplace.
- Current reimbursement policies create incentives for physicians to prescribe high-priced drugs. Payment policies should be redesigned to be neutral to the cost of products.
- Potential unintended consequences on drug access and costs must be carefully considered before implementing requirements related to pricing and payment.

Potential Recommendations Regarding Drug Pricing

- A group of diverse stakeholders—including patients, public and private payers, oncologists, and others—should develop a shared framework of value that includes guiding principles and key elements of value. Metrics for value measurement also should be developed. This type of framework will allow identification of drugs for which value and price are misaligned. The National Academy of Medicine may be an appropriate convening body to foster dialogue. Additional thought will be needed to determine how a value framework could be applied in the context of the U.S. healthcare system.

- Patient-physician decision support tools that include information on various components of drug value (e.g., efficacy, side effects, cost) should be developed and used. Patients and providers also should be urged to contribute data for continued evaluation of treatment regimens.
- The Centers for Medicare & Medicaid Services (CMS) and/or the Patient-Centered Outcomes Research Institute (PCORI) should collect and report data on outcomes among various patient populations treated with various cancer regimens. Identification of underperforming drugs will create downward pressure on prices.

ACCESS TO TREATMENTS

Payment and Reimbursement

- Some groups—mainly outside of the United States—have tested outcomes-based reimbursement strategies in which manufacturers are fully reimbursed if a drug works in a given patient but are reimbursed at a significantly lower rate if a patient does not respond. These strategies have been largely unsuccessful, in part because it is very difficult to monitor patient outcomes. Decisions must be made about which metrics to measure and who should measure them. More sophisticated data systems are needed to make this type of approach workable. Computer science and engineering experts could help build learning healthcare systems for cancer.
- Anti-kickback rules prevent drug manufacturers and payers from discussing drugs prior to FDA approval. Safe harbors that allow some types of conversations may pave the way for alternative reimbursement strategies (e.g., outcomes-based reimbursement).
- State regulations make it difficult for commercial insurance companies to develop value-based benefit designs. However, self-funded insurers have more flexibility to explore creative approaches.

Patient Out-of-Pocket Costs

- Capping patient out-of-pocket costs will inflate insurance premiums. This will result in cost shifting, not overall cost reduction. However, since the number of cancer patients is relatively small compared with the total insured population, the increase in premiums caused by capping out-of-pocket costs for cancer drugs would be spread out over a larger population.
- The Panel should consider issuing a recommendation regarding preferred benefit design. Many payers are considering co-pay caps to help address financial toxicity, at least for insured people. However, these benefit plans often include utilization management features such as prior authorization and step therapy.
- Addressing drug costs or even all direct healthcare costs will not solve the problem of financial toxicity for cancer patients. Half of Americans do not have adequate financial reserves to deal with the indirect costs of a serious health event (e.g., income loss, transportation, child care).

Access to High-Value Care

- Patients should have access to appropriate diagnostic tools (e.g., genetic testing) to ensure they receive treatments best matched to their disease and situation. In some cases, physicians—particularly community oncologists who may not see many patients with any given cancer type—may not be aware of the most up-to-date research. Physicians should be accountable for delivering high-quality care. Continuing medical education and clinical pathways can play important roles in ensuring patients receive evidence-based care.
- Ideally, patients' tumors would be genetically profiled, and this information would inform treatment decisions. However, in many cases, genetic test results are not clearly linked to particular treatment pathways. It is not cost-effective to generate genetic information if physicians do not know what to do with it. More research is needed to identify subpopulations—including those defined by molecular

markers—that are likely and/or unlikely to benefit from various therapies. ASCO’s Targeted Agent and Profiling Utilization Registry (TAPUR) study is designed to determine whether genetic test results correlate with response to various treatments.

- In Nebraska, most patients diagnosed with lymphoma travel to Omaha to receive consultation from internationally renowned experts. A treatment plan is developed, and patients then return home and receive treatment from a community oncologist. This approach provides access to expertise while allowing patients to remain home throughout most of their treatment. A similar approach for lung cancer is being considered in Ohio, wherein patients would receive a diagnostic workup and treatment plan from doctors at The Ohio State University.
- To create a learning healthcare system for cancer, there must be a common set of data elements collected by all physicians. ASCO has created CancerLinQ, which aggregates medical record data to facilitate research. ASCO has found significant gaps in electronic medical records that create challenges for answering even basic questions.
- Unrestricted patient access to all drugs is not ideal. Payers should be able to restrict access to therapies that do not provide high value. As a tradeoff for this restricted access, payers should ensure patients have access to high-value treatments without undue financial burden. Regulations that prevent payers from waiving co-payments and deductibles for high-value regimens should be modified.

FDA Role

- The FDA charge to evaluate drugs based on safety and efficacy should be maintained. Value assessments should occur outside of the regulatory framework.

Potential Recommendations Regarding Access to Treatments

- Review coverage mandates and consider elimination of those that interfere with market competition or are misaligned with evidentiary standards. Patient access to high-value drugs must be maintained, but some mandates are outdated and should be revisited. One option would be to implement mandates based on value assessment (i.e., required coverage for drugs that exceed an established threshold for value) rather than require coverage for all FDA-approved cancer drugs.
- Allow payers to develop flexible benefit designs that enable modification of cost sharing based on an independent assessment of whether a drug’s price is aligned with its value. The National Association of Insurance Commissioners could be asked to allow pilot testing of premium-neutral benefit designs that favor high-value treatments. To avoid unbalanced selection of members, market-wide models could be adopted by multiple carriers. One option is to define high-value treatments as those that receive the “preferred” designation within National Comprehensive Cancer Network Guidelines.
- Create a safe harbor that would allow pharmaceutical companies and payers to communicate, particularly about drugs in the pipeline, to facilitate innovative payment arrangements. Currently, several regulations deter communication and collaboration between manufacturers and payers. FDA recently published guidance regarding Food and Drug Administration Modernization Act Section 114 to clarify allowable communications between manufacturers and payers. If finalized, this guidance will be helpful. However, anti-kickback rules and other policies linking sales prices to payment by federal programs (e.g., Medicaid “best price,” average sales price) also create barriers to collaboration.
- Medicare Part B and Part D should be modified as needed to eliminate incentives to develop and select drugs based on factors other than value (e.g., treatment setting). Changes to Medicare would require an act of Congress. It may be more feasible to charge the Center for Medicare & Medicaid Innovation (CMMI) with pilot testing alternative payment strategies.

- Out-of-pocket catastrophic co-insurance for those covered through Medicare Part D should be capped. This may increase premiums, but it is important to protect patients while other solutions are developed and tested.

COMMUNICATING TREATMENT BENEFITS

- Patients need knowledge about costs of care, but it may not be realistic to expect physicians to deliver this information. Social workers and nurse navigators may not have enough clinical expertise to help patients assess the overall value of a treatment regimen. Some practices employ financial counselors, but these individuals generally focus on insurance coverage-related questions. One participant suggested it may be necessary to create a new position to help address financial issues. This would be an additional expense, and it is not clear whether it would result in overall savings. Another participant stated that, regardless of who helps patients navigate financial issues, physicians should be expected to at least screen for financial toxicity.
- Many patients do not clearly understand the goals of their care. Patients often believe the treatment they receive may be curative, when, in many cases, evidence suggests life will be extended by only a short time. Research is needed to develop and test tools to help patients understand the likely clinical benefits of their treatment options.
- Patients are a valuable resource that should be utilized. Many patients are willing to share their data and/or participate in clinical trials to accelerate drug development. Patients also want to help address financial toxicity and costs of cancer care.
- Dr. Newcomer reported that UnitedHealth's pilot of risk-bearing payments found that about one-third of the reduction in total cancer care cost resulted from reduced hospitalization. In many cases, hospitalization was reduced because patients had high access to their doctors and nurses. Hospitalization of cancer patients often represents a failure. Most side effects can be anticipated and managed in outpatient settings if they are detected early enough.
- In recent years, there has been a shift in where drug development is being done. Academic institutions have become increasingly involved in later-stage drug development, and pharmaceutical companies have begun focusing less on early-stage research. One idea discussed was to explore whether prices of drugs developed largely with federal funds could be limited to ensure taxpayers reap the benefits of their investments.

Potential Recommendations Regarding Communication of Treatment Benefits

- PCORI should evaluate the value and benefit of cancer care across the continuum. Any evaluation should look at total treatment costs, not just costs for drugs.
- The last decade of oncology drug development should be reviewed to determine whether the current system of drug discovery and development adequately promotes innovation. Incomplete knowledge about the biology of cancer creates inherent inefficiency in drug development processes. Continued investment in basic research is imperative.
- Kathy Giusti should be interviewed to identify potentially generalizable lessons from her experience driving development and testing of drugs to treat multiple myeloma. She has helped successfully push forward several drugs in a fraction of the time normally spent on development.

CONCLUSIONS AND CROSS-CUTTING RECOMMENDATIONS

Participants were asked to weigh in on which potential recommendations would have a high impact, propose additional potential recommendations, and/or identify topics that should be addressed in the Panel report.

- The pricing model for oncology drugs is broken—there have been rapid increases in prices without commensurate increases in benefit. Costs have been shifted to patients, who are experiencing financial toxicity. Cost-effectiveness analyses should be used to determine the value of drugs in the United States.
- Solutions to the problem of cancer drug costs must be multifactorial and multisector. There currently is a lack of trust among the various stakeholders, all of whom need to work together to address this problem. Patients, in particular, must be involved. The Panel could propose an ongoing process to continue the productive multi-stakeholder discussions that took place at this workshop.
- Consideration should be given to how to balance individual patient values with societal values.
- Drug prices should be considered within the context of the overall cost of cancer care.
- Increased coverage for preventive care may help reduce overall costs of cancer care by reducing cancer risk.
- More clarity is needed on the goals of insurance coverage, who is responsible for the costs of new drugs, and who reaps the financial rewards.
- The price of a drug should reflect its value. Payers should not be required to cover low-value drugs, and highly effective drugs should be priced higher than less effective drugs.
- Patient cost sharing for high-value drugs should be reduced. To help payers absorb this cost without increasing premiums, coverage mandates for cancer drugs also should be eliminated.
- Regulations must be modified to create a competitive cancer drug marketplace in which payers have leverage to negotiate prices based on a drug's value.
- Regulations should be reformed as needed to enable value-based contracting.
- Incentives should be created for increasing efficiency in drug development. An example of inefficient use of patients and financial resources is the simultaneous conduct of clinical trials on several PD-1 inhibitors.
- Manufacture of generic drugs should be encouraged, and barriers that delay market entry of generic drugs should be addressed (e.g., pay-to-delay tactics, citizen petition filing, secondary patents).
- Incentives should be created to promote use of clinical decision support tools.
- Delivery of futile care, particularly at the end of life, should be reduced.
- Increased price transparency is needed so it is clear who benefits from high drug prices.
- Consideration must be given to the cost of combination therapies, which are becoming common for many cancer types.
- The 340B Drug Pricing Program should be reformed.
- The government should consider exercising existing rights to influence the market in situations in which prices are egregious (e.g., march-in rights, government-use rights).

PUBLIC COMMENT

- Lisa Paradis, Research Analyst for the President's Cancer Panel, shared two tweets posted to [#CancerRxValue](#) during the workshop:
 - Bob Tufts, a former Major League Baseball pitcher who now teaches at New York University, was diagnosed with multiple myeloma in 2009. He tweeted, "Healthcare is too important to be left to the whims of politicians. It must be grounded in the patient and doctor relationship."
 - Another person tweeted, "Only patients and the doctors who see us define value, not some person removed from the bedside."

CLOSING REMARKS

Panel members thanked participants, the co-chair, staff members, the facilitator, and the graphic recorder for their contributions to the workshop. They urged participants to send any additional comments to the Panel office and expressed hope that participants will be willing to provide additional information and insights as the report is developed.

CERTIFICATION OF MEETING SUMMARY

I certify that this summary of the President's Cancer Panel meeting, *Pricing and Payment Strategies for Cancer Drugs: Maximizing Patients' Access to Beneficial Therapies*, held March 27, 2017, is accurate and complete.

Certified by:

Date: 6/16/2017

Barbara K. Rimer, DrPH
Chair
President's Cancer Panel