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Ensuring Patient Access to Affordable Cancer Drugs: Workshop Summary

ISBN
978-0-309-31270-7

74 pages
6 x 9
PAPERBACK (2014)

Sharyl J. Nass and Margie Patlak, Rapporteurs; National Cancer Policy Forum; Board on Health Care Services; Institute of Medicine

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THE NATIONAL ACADEMIES
Advisers to the Nation on Science, Engineering, and Medicine

Ensuring
PATIENT ACCESS
to Affordable
CANCER DRUGS

Workshop Summary

Sharyl J. Nass and Margie Patlak, *Rapporteurs*

National Cancer Policy Forum

Board on Health Care Services

INSTITUTE OF MEDICINE
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THE NATIONAL ACADEMIES PRESS
Washington, D.C.
www.nap.edu

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NOTICE: The workshop that is the subject of this workshop summary was approved by the Governing Board of the National Research Council, whose members are drawn from the councils of the National Academy of Sciences, the National Academy of Engineering, and the Institute of Medicine.

This project was supported by Contract No. HHSN261200900003C between the National Academy of Sciences and the Centers for Disease Control and Prevention and by the American Association for Cancer Research, the American Cancer Society, the American Society for Radiation Oncology, the American Society of Clinical Oncology, the American Society of Hematology, the Association of American Cancer Institutes, C-Change, the CEO Roundtable on Cancer, EMD Serono, Helsinn Healthcare SA, the LIVESTRONG Foundation, the National Comprehensive Cancer Network, Novartis Oncology, the Oncology Nursing Society, and Sanofi Oncology. Additional funding for this workshop was provided by ION Solutions. The views presented in this publication do not necessarily reflect the views of the organizations or agencies that provided support for the activity.

International Standard Book Number-13: 978-0-309-31270-7
International Standard Book Number-10: 0-309-31270-1

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Suggested citation: IOM (Institute of Medicine). 2014. *Ensuring patient access to affordable cancer drugs: Workshop summary.* Washington, DC: The National Academies Press.

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"Knowing is not enough; we must apply.
Willing is not enough; we must do."
—Goethe

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PETER B. BACH (*Chair*), Attending Physician, Center for Health Policy and Outcomes, Department of Epidemiology and Biostatistics, Memorial Sloan Kettering Cancer Center

BARRY FORTNER, President, ION Solutions

PATRICIA A. GANZ, Distinguished Professor, Health Policy & Management and Medicine, UCLA Fielding School of Public Health, David Geffen School of Medicine at UCLA; Director, Cancer Prevention & Control Research, Jonsson Comprehensive Cancer Center

CAROL A. HAHN, Associate Professor of Radiation Oncology, Duke University Medical Center, and Medical Director of Radiation Oncology, Duke Raleigh Hospital, Department of Radiation Oncology

MARTIN J. MURPHY, Chief Executive Officer, CEO Roundtable on Cancer

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RICHARD SCHILSKY, Chief Medical Officer, American Society of Clinical Oncology

DEBORAH SCHRAG, Chief, Division of Population Sciences, Professor of Medicine, Department of Medical Oncology, Dana-Farber Cancer Institute, Harvard Medical School

YA-CHEN TINA SHIH, Associate Professor, Section of Hospital Medicine, Department of Medicine Director, Program in the Economics of Cancer, University of Chicago

JEFFERY WARD, Medical Oncologist, Swedish Cancer Institute, Seattle, WA

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Project Staff

SHARYL J. NASS, Director, National Cancer Policy Forum
SARA THARAKAN, Research Assistant
HANNAH DURING, Senior Program Assistant
CELYNNE BALATBAT, Senior Program Assistant

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NATIONAL CANCER POLICY FORUM[1]

MICHAEL CALIGIURI (*Chair*), CEO, James Cancer Hospital and Solove Research Institute, and Director, OSU Comprehensive Cancer Center, The Ohio State University

PATRICIA A. GANZ (*Vice Chair*), Distinguished Professor, Health Policy & Management and Medicine, UCLA Fielding School of Public Health, David Geffen School of Medicine at UCLA; Director, Cancer Prevention & Control Research, Jonsson Comprehensive Cancer Center

AMY P. ABERNETHY, Associate Professor of Medicine, Duke University School of Medicine, and Director, Duke Cancer Care Research Program, Durham, NC

LUCILE ADAMS-CAMPBELL, Professor of Oncology, Associate Director for Minority Health and Health Disparities Research, Georgetown University Lombardi Cancer Center

KENNETH ANDERSON, Kraft Family Professor of Medicine, American Cancer Society Clinical Research Director, Jerome Lipper Multiple Myeloma Center, Harvard Medical School, Dana-Farber Cancer Institute

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OTIS BRAWLEY, Chief Medical Officer and Executive Vice President, American Cancer Society, Atlanta, GA

CARLTON BROWN, Director of Professional Services, Oregon Nurses Association, and past president, Oncology Nursing Society

SERGIO CANTOREGGI, Chief Scientific Officer and Global Head of Research and Development, Helsinn Group

ROBERT W. CARLSON, Chief Executive Officer, National Comprehensive Cancer Network

WILLIAM S. DALTON, CEO, M2Gen Personalized Medicine Institute, Moffitt Cancer Center, Tampa, FL, and Chair, American Association for Cancer Research Science Policy & Legislative Affairs Committee

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GWEN DARIEN, Executive Vice President, Programs and Services, Cancer Policy Institute, Cancer Support Community

WENDY DEMARK-WAHNEFRIED, Associate Director for Cancer Prevention and Control, University of Alabama at Birmingham Comprehensive Cancer Center

CAROL A. HAHN, Associate Professor of Radiation Oncology, Duke University Medical Center, Durham, NC, and Medical Director of Radiation Oncology, Duke Raleigh Hospital, Raleigh, NC, and Clinical Affairs and Quality Council Chair, American Society for Radiation Oncology

LORI HOFFMAN HOGG, Cancer Program Director, Albany Stratton VA Medical Center

THOMAS J. KEAN, President and CEO, C-Change, Washington, DC

SAMIR N. KHLEIF, Director, Georgia Health Sciences University Cancer Center, Georgia Regents University Cancer Center

MICHELLE M. LE BEAU, Arthur and Marian Edelstein Professor of Medicine, Director, Comprehensive Cancer Center, University of Chicago, IL, and President, Association of American Cancer Institutes

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GRETA MASSETTI, Associate Director for Science, Division of Cancer Prevention and Control, Centers for Disease Control and Prevention

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MARTIN J. MURPHY, Chief Executive Officer, CEO Roundtable on Cancer, Durham, NC

RICHARD PAZDUR, Director, Office of Oncology and Hematology Products, Food and Drug Administration

STEVEN PIANTADOSI, Phase One Foundation Endowed Chair and Director, Samuel Oschin Comprehensive Cancer Institute, Cedars-Sinai Medical Center, Los Angeles, CA

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ANDREW SCHIERMEIER, Senior Vice President, Head of Global Oncology, Merck Serono

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RICHARD SCHILSKY, Chief Medical Officer, American Society of Clinical Oncology

DEBORAH SCHRAG, Chief, Division of Population Sciences, Professor of Medicine, Department of Medical Oncology, Dana-Farber Cancer Institute, Harvard Medical School

ADRIAN SENDEROWICZ, Vice President, Global Regulatory Affairs, Sanofi Oncology

YA-CHEN TINA SHIH, Director, Program in the Economics of Cancer, University of Chicago, IL

ELLEN V. SIGAL, Chair and Founder, Friends of Cancer Research, Washington, DC

STEVEN STEIN, Senior Vice President, U.S. Clinical Development and Medical Affairs, Novartis Oncology, East Hanover, NJ

RALPH WEICHSELBAUM, Daniel K. Ludwig Professor and Chair, Department of Radiation Oncology, and Director, Ludwig Center for Metastasis Research, University of Chicago Medical Center, IL

National Cancer Policy Forum Staff

SHARYL J. NASS, Forum Director and Interim Director, Board on Health Care Services (from June 2014)

SARA THARAKAN, Research Assistant

HANNAH DURING, Senior Program Assistant

PATRICK ROSS, Senior Program Assistant (from September 2014)

CELYNNE BALATBAT, Senior Program Assistant

PATRICK BURKE, Financial Associate

ROGER HERDMAN, Director, Board on Health Care Services (until June 6, 2014)

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This workshop summary has been reviewed in draft form by individuals chosen for their diverse perspectives and technical expertise, in accordance with procedures approved by the National Research Council's Report Review Committee. The purpose of this independent review is to provide candid and critical comments that will assist the institution in making its published workshop summary as sound as possible and to ensure that the workshop summary meets institutional standards for objectivity, evidence, and responsiveness to the study charge. The review comments and draft manuscript remain confidential to protect the integrity of the process. We wish to thank the following individuals for their review of this workshop summary:

MEGHAN BUZBY, International Myeloma Foundation
THOMAS FEELEY, The University of Texas MD Anderson Cancer Center
CAROL S. PALACKDHARRY, Healthcare Consultant and Medical Oncologist
LOWELL E. SCHNIPPER, Harvard Medical School and Beth Israel Deaconess Medical Center

Although the reviewers listed above have provided many constructive comments and suggestions, they did not see the final draft of the work-

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shop summary before its release. The review of this report was overseen by **JOSEPH P. NEWHOUSE,** Harvard University. Appointed by the Institute of Medicine, he was responsible for making certain that an independent examination of this report was carried out in accordance with institutional procedures and that all review comments were carefully considered. Responsibility for the final content of this report rests entirely with the rapporteurs and the institution.

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Acknowledgments

Support from the many annual sponsors of the Institute of Medicine's National Cancer Policy Forum is crucial to the work of the Forum. Current sponsors include the Centers for Disease Control and Prevention, the National Cancer Institute, the American Association for Cancer Research, the American Cancer Society, the American Society for Radiation Oncology, the American Society of Clinical Oncology, the American Society of Hematology, the Association of American Cancer Institutes, the Cancer Support Community, C-Change, the CEO Roundtable on Cancer, EMD Serono, Helsinn Healthcare SA, the LIVESTRONG Foundation, the National Comprehensive Cancer Network, Novartis Oncology, the Oncology Nursing Society, and Sanofi Oncology. Additional funding for this workshop was provided by ION Solutions.

The Forum wishes to express its gratitude to the expert speakers whose presentations helped define the current challenges and opportunities for ensuring patient access to affordable cancer drugs. The Forum also wishes to thank the members of the planning committee for their work in developing an excellent workshop agenda.

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BOXES

FIGURES

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Ensuring Patient Access to Affordable Cancer Drugs: Workshop Summary

TABLE

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Acronyms

ACA	Patient Protection and Affordable Care Act
ANDA	Abbreviated New Drug Application
ASCO	American Society of Clinical Oncology
ASP	average sales price
CDC	Centers for Disease Control and Prevention
CMS	Centers for Medicare & Medicaid Services
DSH	disproportionate share hospital
EMR	electronic medical record
FDA	Food and Drug Administration
HRSA	Health Resources and Services Administration
ICER	incremental cost effectiveness ratio
IOM	Institute of Medicine
IT	information technology
MDACC	The University of Texas MD Anderson Cancer Center
MedPAC	Medicare Payment Advisory Committee

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NCPF	National Cancer Policy Forum
NHS	National Health Service
NICE	National Institute for Health Care Excellence
PCORI	Patient-Centered Outcomes Research Institute
QALY	quality-adjusted life year

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Workshop Summary

INTRODUCTION

In recent years, patients' out-of-pocket costs for cancer care have been rising rapidly. These costs include health insurance deductibles, coinsurance, and co-payments for covered services, as well as services that are not covered by insurance. Many cancer patients are especially vulnerable financially because their illness and/or treatment impedes their ability to work, with some patients losing employment altogether. Even with insurance, cancer patients often experience financial hardships, such as going into debt, depleting all assets to pay for cancer treatment, and personal bankruptcy. In 2012, the National Cancer Policy Forum (NCPF) of the Institute of Medicine (IOM) held a workshop to discuss the affordability of cancer care and potential actions to improve affordability (IOM, 2013b). Following that workshop, the NCPF has developed a series of workshops to delve more deeply into the cost and affordability of different components of cancer care. The goals of these workshops are to encourage dialogue among stakeholders, raise awareness of the issues, and to generate ideas for potential solutions to existing challenges.

Although many elements contribute to the cost of cancer care, one important component is the cost of new cancer drugs, which has been escalating rapidly in recent years. Many cancer patients have large out-of-pocket expenses for the drugs used to treat their disease, often accruing thousands of dollars in drug expenses annually. For some patients with inadequate

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insurance coverage, high drug prices simply put cancer treatment out of reach. At the same time, shortages of older but critical cancer drugs have become commonplace in recent years, and access to community cancer care has become more limited as many private practices have migrated to hospitals, who typically charge more for their services. Thus, to improve cancer care, there is a need to consider patient access to appropriate cancer drugs and other treatments broadly. To explore the issue of cancer drug costs and patient access to affordable, appropriate drug therapies, the NCPF convened a workshop[1] on ensuring patient access to affordable cancer drugs on June 9, 2014, in Washington, DC. Affordability was considered from both the individual and societal perspectives. The workshop featured discussion panels as well as invited presentations from clinicians, researchers, representatives from the health insurance and pharmaceutical industries, and patient advocates.

Workshop sessions fostered dialogue among speakers and participants on topics that included

- Trends in oncology care, such as escalating drug prices, consolidation of private practices into hospital partnerships, drug shortages, and the financial toxicity of rising out-of-pocket costs of cancer treatment;
- Policy factors, such as cancer drug reimbursement and cost-sharing policies (e.g., co-pays and coinsurance), Medicare reimbursement policies and legislative limitations on those policies, and state laws prohibiting restrictions on oncology drug prescribing; and
- Ways to counter the rising costs of cancer care. Suggestions discussed included value-based insurance design and drug pricing, episode-based reimbursements, and incentives for patients and their physicians to opt for lower cost care without compromising the quality of that care.

This report is a summary of the presentations and discussions at the workshop. A broad range of views and ideas were presented and a summary of

[1]This workshop was organized by an independent planning committee whose role was limited to the identification of topics and speakers. This workshop summary was prepared by the rapporteurs as a factual summary of the presentations and discussions that took place at the workshop. Statements, recommendations, and opinions expressed are those of individual presenters and participants; are not necessarily endorsed or verified by the Institute of Medicine or the National Cancer Policy Forum; and should not be construed as reflecting any group consensus.

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suggestions from individual participants is provided in Box 1. The workshop Statement of Task and agenda can be found in the Appendix. The speakers' biographies and presentations (as PDF and audio files) have been archived at http://www.iom.edu/Activities/Disease/NCPF/2014-JUN-09.aspx.

NEW LANDSCAPE OF CANCER CARE

Several speakers described the new landscape of cancer care that is marked by skyrocketing costs, drug shortages, and loss of many community oncology practices as they migrate to hospitals. Workshop participants discussed the role that many different stakeholders could take in addressing these challenges, including patients and care providers, health care payers, and pharmaceutical manufacturers. Barry Fortner, president of ION Solutions, noted that the cost of cancer care has outpaced general inflation and much of the costs in other areas of health care, with projections that cancer costs will continue to rise over the next decade (Mariotto et al., 2011). Michael Kolodziej, the national medical director for oncology solutions at Aetna, presented data showing that cancer care is at the leading edge of the trend for rising medical costs, increasing at two to three times the rate of other health care costs (see Figure 1). The average monthly cost of cancer drug therapy has increased from $100 in 1965 to $10,000 in 2013 (see Figure 2). Mark Hartstein, director of the hospital and ambulatory policy group at the Centers for Medicare & Medicaid Services (CMS), cited a report indicating that the largest payments for Medicare's Part B drug spending went to hematology, oncology, medical oncology, and urology, which collectively made up more than half of this amount (MedPAC, 2003). This same report found that total Medicare spending on drugs increased from $400 million to $7 billion from 1992 to 1999, increased by another $1 billion in 2000, and then increased an additional 26 percent (to $1.5 billion) between 2001 and 2002. The report attributed the growth to the increased volume of new and more expensive medications substituted for older therapies.

A number of new chemotherapies are also coming on the market that are administered orally rather than intravenously and are usually more expensive than intravenous versions that are off patent, reported Yousuf Zafar, a medical oncologist with Duke Medicine. In addition, the cost per pill of oncology drugs has been increasing. Between 2007 and 2014, the cost of Tarceva went from $100 to $200, Sprycel increased by 130 percent, and Gleevec increased by 158 percent (Langreth, 2014).

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BOX 1
Suggestions Made by Individual Workshop Participants

Empower and incentivize patients to choose high-value treatment options

- Engage patients in discussions about the costs and potential benefits of their care when considering a treatment plan (Yousuf Zafar, Duke Medicine)
- Increase use of specialty pharmacies that offer supportive care to patients undergoing chemotherapy (Lee Newcomer, UnitedHealth)
- Use cost sharing to encourage patients to select high-performing providers and care settings (Mark Fendrick, University of Michigan; Eric Hammelman, Avalere Health)
- Use value-based insurance designs in which the cost-sharing level depends on the clinical benefit, not acquisition price, of the service (Mark Fendrick; Patricia Danzon, University of Pennsylvania)
- Reduce cost sharing in accordance with patient- or disease-specific characteristics (Mark Fendrick)
- Relieve patients from cost sharing if they fail to respond to a lower cost medication (Mark Fendrick)
- Increase transparency in the 340B program to ensure that it is helping vulnerable populations as intended (Peter Bach, Memorial Sloan Kettering Cancer Center; Rena Conti, University of Chicago)

Empower and incentivize physicians to use high-value treatment options

- Design electronic medical records to provide information on the evidence base and cost for a treatment (Mark Fendrick)
- Encourage greater use of practice guidelines and more consistency among payers regarding the guidelines used (Bruce Gould, Community Oncology Alliance)
- Establish reimbursement rates based on data from relevant patient populations (Kevin Olson, Providence Hospital)
- Reform reimbursement practices to replace the traditional "buy and bill" way of doing business in oncology (Thomas Feeley, MD Anderson Cancer Center; Bruce Gould; Michael Kolodziej, Aetna; Jeffrey Peppercorn, Duke Medicine)
  - Reimburse the chemotherapy administration fee separately from the drug fee (Bruce Gould, Michael Kolodziej)
  - Adequately compensate oncologists for the complex and time-consuming care they offer patients (Jeffrey Peppercorn)
  - Use bundled payments tied to metrics to incentivize efficient care and good patient outcomes (Thomas Feeley)

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- Educate physicians on cost-cutting strategies such as prescribing generics, offering less expensive therapeutic alternatives, and offering discount cards (Jeffrey Peppercorn, Yousuf Zafar)
- Provide guidance to eliminate care for which the evidence convincingly shows a lack of value or potential harm to patients (Mark Fendrick, Eric Hammelman)
- Develop policies that incentivize oncologists to stay in private practice (Bruce Gould, Jeffrey Peppercorn)
  - Provide payment parity for administrative services for the hospital versus the physician office (Bruce Gould, Jeffrey Peppercorn)
  - Remove the sequestration cuts to Medicare Part B drugs (Bruce Gould)
  - Remove prompt pay discounts from the calculation of the ASP (average sales price) (Jeffrey Peppercorn)

Develop policies that counter price escalation for cancer drugs

- Use value-based pricing for cancer drugs, in which higher prices are paid for drugs showing better treatment outcomes, to discourage the use of ineffective but costly therapies (Patricia Danzon, Michael Kolodziej)
- Make the cancer care market more competitive (Kalipso Chalkidou, National Institute for Health and Clinical Excellence; Rena Conti; Patricia Danzon; Peyton Howell, AmerisourceBergen)
- Allow the Centers for Medicare & Medicaid Services to negotiate prices and consider cost-effectiveness of drugs (Jeffrey Peppercorn, Yousuf Zafar)
- Put more information in the public domain to inform payment decisions (Kalipso Chalkidou)
- Reimburse less for new drugs until sufficient evidence has been gathered on patient outcomes (Jeffrey Peppercorn)
- Give pharmaceutical firms a guaranteed price on the market in place of patent protection for long periods of time (Lee Newcomer)

Address drug shortages

- Accelerate or prioritize regulatory support for low-cost cancer drugs or those with supply issues (Peyton Howell, Yousuf Zafar)
- Adjust reimbursement for generics to a higher rate (Peyton Howell, Jeffrey Peppercorn)
- Adjust drug reimbursement levels more quickly in response to price changes to help stabilize supply and demand (Peyton Howell, Yousuf Zafar)
- Consider new ways in which the federal government could foster production of generic drugs to avoid shortages (Jeffrey Peppercorn)

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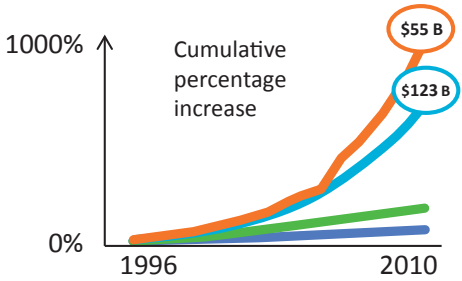

	Annual Increase
Cancer Drugs	20%
Cancer Medical	12-18%
Health Care	9%
US GDP	3%

FIGURE 1 Increase in cancer care and drug costs relative to overall health care costs. Cancer is the most costly medical item and is increasing at 2-3 times the rate of other costs in health care.
NOTE: CY = calendar year; US GDP = United States gross domestic product.
SOURCES: Kolodziej presentation, June 9, 2014; 2010 CY claims; commercial and Medicare; all funding; http://www.cancer.gov/newscenter/newsfromnci/2011/Cost Cancer2020 (accessed August 20, 2014).

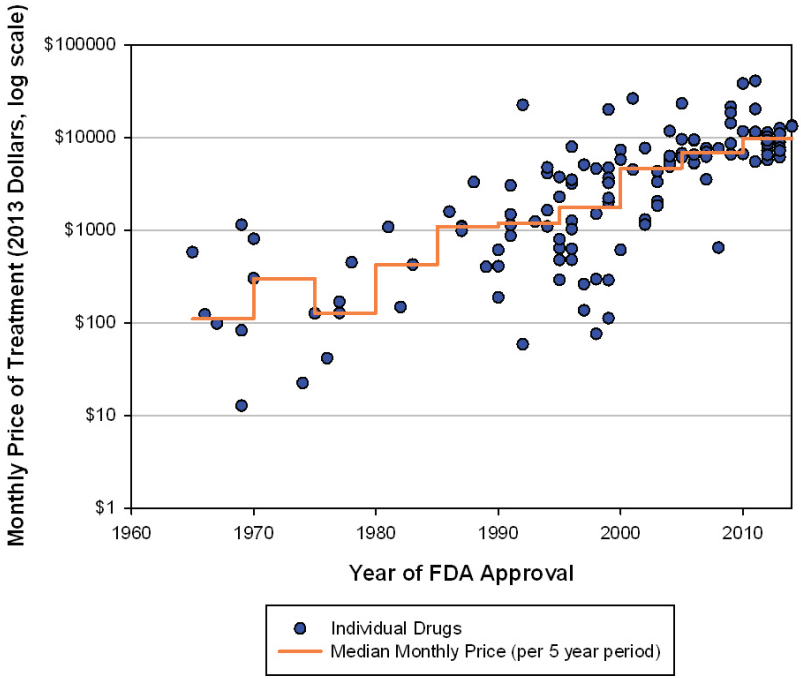

FIGURE 2 Monthly and median costs of cancer drugs at the time of the Food and Drug Administration (FDA) approval (1965-2014). Median monthly price has increased 100-fold since 1965.
SOURCES: Bach presentation, June 9, 2014; adapted from Bach, 2009.

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Because of their skyrocketing cost, drugs now comprise 35 percent of the cost of cancer care, said Eric Hammelman, vice president at Avalere Health, up from 25 percent just a few years ago. Kolodziej gave the specific example of imatinib (Gleevec), which was developed to treat chronic myelogenous leukemia. Initially offered a decade ago at the price of $30,000 per year, treatment with this drug now costs more than three times as much (Dusetzina et al., 2014). "That's the new landscape," he said.

High Out-of-Pocket Expenses

Some insured patients go bankrupt before they reach the cap on out-of-pocket expenses, when their cancer care bills are paid in full by private or federal insurance plans (Dusetzina et al., 2014). Patient out-of-pocket expenses for health care overall average $325 per year, with an expected increase to $450 by 2022, Zafar noted. However, cancer patients often have much greater than average out-of-pocket expenses, with one study finding they may pay $4,000 to $5,000 per year for their cancer care (Bernard et al., 2011). One-third of those expenses are due to their prescription drugs, another one-third to other ambulatory care, including physician fees and outpatient procedures, and the remainder to inpatient care and other costs (Bernard et al., 2011). Patients who lack adequate insurance coverage are at much greater risk for high out-of-pocket expenses. Zafar gave the example of one of his patients who was diagnosed with rectal cancer. This patient was employed and insured, but lacked prescription drug coverage. He had incurred thousands of dollars of out-of-pocket drug expenses during the course of his treatment with oral capecitabine.

Another example was cited by Lee Newcomer, senior vice president at UnitedHealthcare with strategic responsibility for oncology, genetics, and women's health. He described the case of an Oregon farmer who developed colon cancer and wanted to receive oral chemotherapy so he could continue to work during the harvest season. Unfortunately, the inexpensive health care plan he purchased only paid for half of this drug therapy, so his out-of-pocket expenses totaled $50,000. "He got what he paid for," Newcomer stressed, noting that if the farmer had purchased a plan with better benefit coverage, he would not have amassed such a large cost of care for which he had to pay. But studies show that one-third to 40 percent of patients do not feel a $50 co-payment for a $5,000 drug is value worth their money, either because they cannot afford it or because "they are thinking as a consumer and not as a patient," Newcomer said. "The Oregon farmer is thinking 'I

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don't want to pay for my coverage, but it better be very good if I ever get sick.'"

However, Mark Fendrick, director, University of Michigan Center for Value Based Insurance Design, noted that in his work with the underserved, even a very low cost share can be a hardship, and reducing patient cost to zero substantially increases treatment adherence. Shelley Fuld Nasso, senior director of policy at the National Coalition for Cancer Survivorship, added, "What may seem like a small co-pay is a lot to some people—it is not just the equivalent of 10 Starbucks coffees, but whether they have food for themselves or whether they can buy school supplies for their kids. We do not want co-pays they cannot afford," she said.

Out-of-pocket costs also include paying for the care given by specialty providers not within the patient's insurance network of physicians, noted Brian Rosen, chief policy and advocacy officer of the Leukemia & Lymphoma Society. Such costs do not count toward a patient's deductible.

The rise in out-of-pocket cancer care costs has been magnified by the increase in cost sharing by various insurance plans. Zafar showed that insurance premiums increased between 1999 to 2013 by 182 percent, with the worker contribution to premiums going up by nearly 200 percent (see Figure 3). This study also found that between 2006 and 2013, the average deductible paid by patients has nearly doubled (Kaiser Family Foundation and Kaiser Health Research and Educational Trust, 2013). "Insurance has become more expensive, and the amount that patients pay before their insurance takes over has also increased because deductibles have increased as well," Zafar noted. Peyton Howell, senior vice president and president, global sourcing and manufacturer relations for AmerisourceBergen added, "The cost crisis is really due not just to patients who are not insured, but also due to patients who are underinsured." Bruce Gould, vice president of the Community Oncology Alliance, stressed, "A lot of the administrative burden of our practice is getting these underinsured or uninsured patients access to care through co-pay foundations and foundations that supply free drugs, etc."

Kolodziej said that with the Patient Protection and Affordable Care Act (ACA), "the maximum out-of-pocket expense is reached in the first month of treatment, and deductibles and coinsurance become irrelevant. Member responsibility is capped and cost of this treatment becomes society's responsibility." However, according to Zafar, the ACA is not expected to significantly reduce out-of-pocket cancer care costs. He noted that most enrollees have signed up for silver plans, which would require a family of four with

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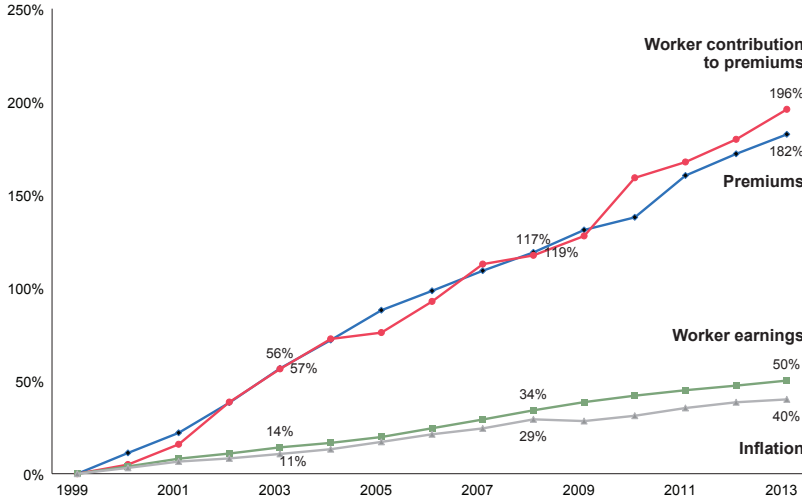

FIGURE 3 Health insurance costs are increasing rapidly. Health insurance premiums, and worker contributions to those premiums, have been increasing much faster than worker earnings or inflation.
SOURCES: Zafar presentation, June 9, 2014; Kaiser Family Foundation and Kaiser Health Research and Educational Trust, 2013.

an annual income of about $47,000 to pay about $10,000 out-of-pocket costs before they would reach the cap limit. "I'm not really certain that out-of-pocket costs for cancer care will necessarily decrease with the Affordable Care Act," Zafar said, "and as a result, patients are having difficulty paying their medical bills." Data suggest that between one out of three and one out of five patients report difficulty paying their medical bills (Kaiser Family Foundation and Kaiser Health Research and Educational Trust, 2013).

High out-of-pocket medical expenses are not just a financial issue, but can adversely affect patient and family well-being. Zafar did a study of underinsured cancer patients and found that about half were spending their savings to help pay for their cancer care. About half also reported cutting back on food and clothing, with 17 percent reporting selling property to cover their expenses. About two-thirds reported cutting back on vacation or leisure activities because of the cost of their cancer treatment. "One woman in the study we talked to said her vacation was the only time after her kids left home in which her family could get together, and now, because of the cost of her cancer treatment, that was gone," Zafar said (Zafar et al., 2013).

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Another study found a link between cancer diagnosis and risk of bankruptcy (Ramsey et al., 2013).

High out-of-pocket costs also seem to adversely affect patients' care, mainly due to a lack of treatment adherence because of unaffordability, Zafar reported. One study found patients with a higher co-payment for a cancer drug (about $30 per month) had a 42 percent higher likelihood of non-adherence (Dusetzina et al., 2014). Another study found that lower co-payment for imatinib led to better patient treatment adherence, even though the median co-payment amount was only $40 (Dusetzina et al., 2014). "Small out-of-pocket expenses have a large effect on discontinuation so we have an adherence problem," stressed Kolodziej. He added that given the importance of treatment adherence to patient outcome, higher cost sharing with patients is likely to reduce the effectiveness of treatment. Zafar added, "We're seeing a growing list of financial toxicities due to out-of-pocket cost," with the lack of adherence suggesting that poorer cancer-related outcomes are also likely for some curable cancers, although no study has yet to show this.

Migration of Private Practices to Hospitals

Out-of-pocket costs are also increasing due to the rising migration of community oncology practices to hospitals. Just within 2013, 288 clinics closed, Gould noted, and four recent studies reveal that the cost of care, including both radiation therapy and chemotherapy, is higher in the hospital outpatient department compared to the oncologist's office. One study of breast, lung, and colon cancers, for example, found that in a hospital setting, care for adjuvant and metastatic episodes was 28 to 52 percent higher on average (Milliman, 2013). Another study found that the cost of care in a hospital outpatient setting costs Medicare $6,500 more per year per beneficiary (the patient's out-of-pocket cost was $650 per year), which translates into a 14 percent cost differential between the two sites of service (Milliman, 2011).

Gould added that over the past 10 years, hospitals have steadily been paid more for their chemotherapy administrative services, whereas practices have experienced a steady decline (see Figure 4).That could be due to many payers having limited leverage with hospitals because in many communities there is only one hospital, "so if they negotiate hard on the oncology services, they'll be charged higher fees somewhere else in the basket of goods," Gould said.

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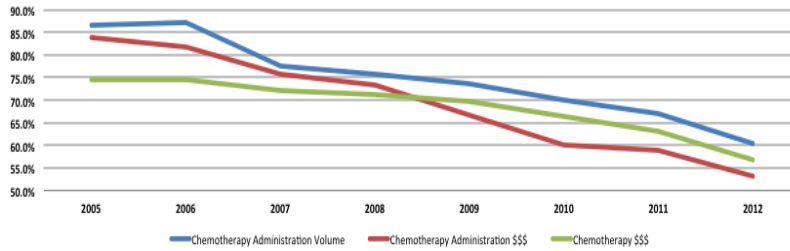

FIGURE 4 Community cancer care share versus outpatient hospital. Over the past 10 years, hospitals have steadily been paid more for their chemotherapy administrative services, whereas practices have experienced a steady decline in patient volume and reimbursements.
SOURCES: Gould presentation, June 9, 2014; Community Oncology Practice Impact Report, Community Oncology Alliance, July 2013; Results of Analyses for Chemotherapy Administration Utilization and Chemotherapy Drug Utilization, 2005-2011 for Medicare Fee-for-Service Beneficiaries, The Moran Company, May 2013.

Hammelman added that physicians are required by Medicare to submit a code for their services and are reimbursed based on that fee code, whereas hospitals are not scrutinized as carefully and instead of submitting a code, they are reimbursed a percentage of what they charge, regardless of the reason for the charge. That percentage varies between 50 and 70 percent, depending on the hospital's market power. "This is not an efficient payment system. When you tell people you're going to pay them some percentage of their cost, naturally costs go up and that's what happened," Hartstein noted. When Hammelman compared the mark-ups on the same chemotherapy drugs at the same dose provided by hospitals versus that of private practices, he found hospitals have a mark-up three to five times greater, which can result in as much as a $6,200 difference for services provided in a hospital versus in private practice.

In 2005, about 80 percent of oncology patients received their care in private practice, but now only between 50 to 60 percent do, according to Gould, who noted that during a recent 15-month period, 469 practices merged with hospitals (Community Oncology Alliance, 2013; The Moran Company, 2013).

The closure of community clinics not only affects patients' pocketbooks, but their quality of life. Gould quoted one breast cancer survivor who stated, "When you're sick and you have to travel 30 miles to see the

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doctor, it feels like 300 miles." The greater number of hospital-based oncology practices also is driving up the costs of insurance premiums, according to Hammelman. "As more patients start to shift to hospitals and their higher costs, all of our premiums go up. Even if you're not on therapy right now, you're paying for this at the moment as this trend continues to happen," he stressed.

FACTORS INFLUENCING CANCER CARE ACCESS AND COSTS

Workshop participants discussed a number of factors affecting access to cancer drugs and increased costs, including

- Drug pricing practices and the increased cost of developing new drugs;
- Shortages of older generic drugs;
- Consolidation of practices; and
- Reimbursement incentives that foster the use of higher cost drugs and the shift of site of care from the community to the hospital setting.

Alex Bastian, head of the San Francisco office of Gfk Bridgehead, provided an overview of drug pricing practices and the factors that influence those practices (see Box 2). He said that regulatory factors can influence how quickly a drug enters and grows a market. Oncology drugs comprise about one-third of all drugs given a fast track approval by the Food and Drug Administration (FDA), and the quicker time to market via this regulatory pathway boosts the potential value of the drug, he said. Once a drug enters the market in the United States, it usually becomes widely available, unlike in Europe, where there can be more limited distribution until there is a build-up of evidence on the value of the drug (FDA, 2014). But a number of U.S. state laws aim to reduce the cost of cancer pharmaceuticals for patients, Bastian reported. These include oral parity laws, cost-sharing and out-of-pocket spending limits, specialty tier laws, step-edit or "fail first" therapy laws, and requirements for coverage of care in clinical trials (Abbott, 2014; Brooker, 2013; Global Healthy Living Foundation, 2010; NCI, 2012).

The price of a drug depends in part on how many patients can use it, and the drug's market share of that patient volume. The larger the volume, the lower the price. Bastian noted that as cancer therapies have been

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BOX 2
What Determines Drug Value and Price?

For pharmaceutical companies, the value of a drug depends on a number of factors, including

- How big the market is for the drug
- How long it takes to grow that market and reach peak sales
- How long the drug has a period of exclusivity on the market
- How long a drug is on the market

Typically manufacturers use three basic strategies when pricing their drugs:

- Maximize their profits by trading greater volume for a higher price.
- Charge the price at which the largest volume of consumers will have access to the drug, which is typically done for "me-too drugs" or combination products.
- Charge the maximum price achievable when there is a very small set of patients that can use the drug, as is the case for orphan drugs.

SOURCE: Bastian presentation, June 9, 2014.

targeted with more precise indications, the potential market has become smaller. Consequently, the market volume for each cancer drug, that is, the number of patients who can use it, has halved or decreased by one-third between 2001 and 2013, about the same period of time during which the price of cancer drugs has skyrocketed (Meyrowitz et al., 2014). "We're seeing this focus on the smaller, more niche patient population," Bastian stressed. "One of the paradoxes may be that people expect the same price for smaller populations, which is essentially carving off half of the pie," Bastian noted. In addition, there are more treatment options for each type of cancer, which increases complexity, decreases addressable populations, and adds pressure to price competitively and/or contract to secure broad access, Bastian said.

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Typically manufacturers use three basic strategies when pricing their drugs. One is to maximize their profits by trading greater volume for a higher price. Another is to charge the price at which the largest volume of consumers will have access to the drug, which is typically done for "me-too drugs" or combination products. The third is to charge the maximum price achievable when there is a very small set of patients that can use the drug, as is the case for orphan drugs.

The last strategy is increasingly being used as molecular profiles parse cancer patient populations into smaller groups, Bastian noted. In 2013, manufacturers launched twice the number of drugs with associated biomarkers compared to 2001, he reported (Meyrowitz et al., 2014). As a result, the average size of the eligible patient population for oncology drugs has been declining. "For example, if you had an eligible population of about 6,000 patients in 2001, today that would be around 3,000 patients. You double the price, but halve the volume of these drugs." An extreme example of this phenomenon is Crizotinib, which targets the 6 percent of lung cancer patients with mutations in the ALK gene. "That's a very small and niche patient population from which you have to recoup your cost," Bastian said.

Zafar suggested that the cause of the dramatic rise in cancer drug prices in recent years is due to biologic agents replacing traditional cytotoxic agents. Biologics cost more to develop and produce. In 2003, biologics made up about 10 percent of the oncology market. But by 2013 they made up nearly half of that market (Rickwood and Di Biase, 2014). Another study cited by Bastian found that biologics currently comprise 55 percent of oncology drugs used clinically (Schumock et al., 2014). In addition, as previously mentioned, a number of oral versions of traditional chemotherapies are coming on the market. These tend to be much more expensive than the same drug administered intravenously.

Kalipso Chalkidou, founding director, National Institute for Health and Clinical Excellence International, pointed out that the current high costs of developing drugs, which include the costs of developing drugs that fail to make it to the market, is driving up drug prices. "We're faced with a high failure rate, and therefore the cost of success is very high," she said. That is compounded by a limited number of manufacturers compared to the demand for the drug and willingness to pay for expensive new drugs. "There is little control on the demand side. The U.S. market is perhaps doing us all a disservice by being a bit lenient when it comes to paying for a new pharmaceutical product," she said. Chalkidou stressed that because of U.S. payers' willingness to pay the high prices of drugs, there is a disconnect

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between return on investment and value for money in the pharmaceutical industry.

Drug Shortages

The challenges in ensuring patient access to cancer drugs is compounded by drug shortages, which have been increasing for commonly used cancer medications, Howell noted. One recent study showed that 82 percent of oncologists had experienced a shortage in their drug supplies in the past 6 months, with many reporting more frequent drug shortages (Shuman and Emanuel, 2013). The majority of strategies that oncology practices and hospitals use to respond to such drug shortages ultimately results in paying more for the needed drugs (see Table 1) (Gogineni et al., 2013).

Howell described a number of factors that influence drug shortages, including manufacturing quality challenges; complexity, cost, and regulation of complex manufacturing processes; limited markets combined with low sale costs for some drugs; and reimbursement constraints (see Figure 5). She pointed out that many of the drug shortages experienced are not due to a quality issue related to the product itself, but due to a changing standard for the drug. "Sometimes it relates to a collaboration gap between the regulatory environment and those manufacturers that really need to resolve a compliance issue and gets compounded by the fact that it's not given priority," she said.

Drug manufacturing has also gotten more complex, with more components being added to the process, Howell said. For example, most cancer drug shortages are for injectable generics, which, due to their complexity to manufacture, require more investment by the manufacturer. But the low-

TABLE 1 Response of Oncologists to the Shortage of Chemotherapy Drugs

Adaptation	%
Switched chemotherapy regimen	78.4
Substituted a drug partway through treatment regimen	76.7
Delayed treatment	43.2
Excluded some patients	36.9
Omitted doses	29.0
Reduced doses	19.9
Referred patients to another practice	16.5

SOURCES: Howell presentation, June 9, 2014; Emanuel et al., 2013.

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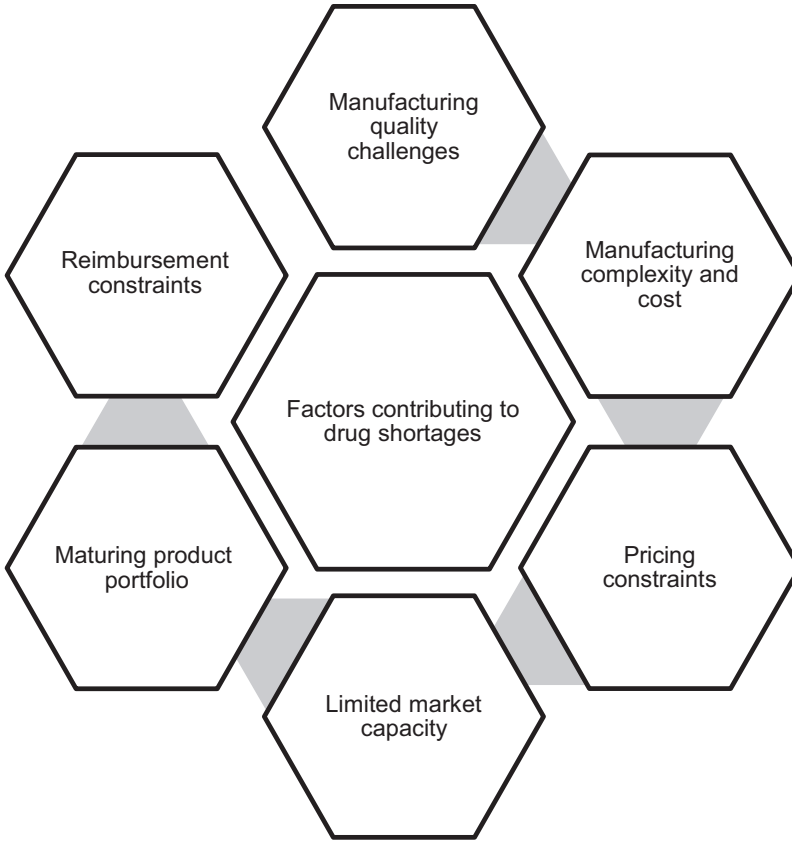

FIGURE 5 Factors contributing to drug shortages.
SOURCE: Howell presentation, June 9, 2014.

priced drug that results from this process is not an attractive investment, so it is common to only have one or two manufacturers for such lifesaving products, Howell said. "We have a really high-cost piece when a product first launches [when it is protected by patent], and then this dramatic reduction, such that people exit the space from a manufacturing perspective," she stressed.

Further compounding drug shortages are the delays due to acquiring approval from the FDA for an Abbreviated New Drug Application (ANDA) for a generic drug and the slow speed of inspections needed for those approvals. "It can take at least 30 months to be able to get through just the

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normal requirements of the best case scenario to bring a product to access," Howell said, and added that there is no way to prioritize the approval process for products with supply issues. That is a challenge for lifesaving cancer drugs that often prompt physicians to prescribe more expensive alternatives.

Reimbursement Constraints and Incentives

Reimbursement constraints contribute to drug shortages by limiting the normal laws of supply and demand, according to Howell, especially for low-cost generic drugs whose mark-up margin is so small (Link et al., 2012). "The band of reimbursement, because it's fixed in terms of those margins being very low, results in pennies on a very low-cost product. These reimbursement constraints are more sensitive on your low-cost products so we have a disincentive to use them," Howell said.

Reimbursement policies also incentivize oncologists to prescribe high-cost drugs and to be hospital based. Jeffrey Peppercorn, associate professor of medicine, Division of Medical Oncology at Duke University Medical Center, pointed out that after the Medicare and Prescription Drug Improvement and Modernization Act of 2003 was enacted, physicians were reimbursed the average sales price (ASP) of a drug plus 6 percent, which was reduced to 4 percent after the sequestration. This incentivizes the use of more expensive drugs, Peppercorn said. For example, he noted that reimbursement for a generic version of paclitaxel, which costs about $312 per dose and requires 6 months of treatment, would provide practices with only the cost of the drug plus $336 to keep their practices running, and pay nurses and other staff. But if physicians prescribe Abraxane, which is the more expensive version of paclitaxel and costs $6,000 per dose, they will pocket nearly $3,000 from a standard course of therapy. In addition, the ASP is not kept up to date, and it does not adequately cover administration costs, including monitoring after giving a patient a dangerous chemotherapy drug. Some offices also may not be privy to volume discounts on a drug given to others that could help sustain their practices, and thus are inspired to close their private practices and work for hospitals instead, Peppercorn noted. "Financial incentives matter," he stressed.

Peppercorn also pointed out a study by Rena Conti, assistant professor of health economics at the University of Chicago, that shows the steep drop-offs in price, as much as 90 percent, when the patent for a high-priced drug expires. This can foster a drop in the income of oncology practices. One study suggested that when cancer drugs go off patent, oncologists shift to

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using more expensive drugs to maintain their income, or prescribe more chemotherapy to defend their revenue (Jacobson et al., 2006). For example, Conti found an 18 percent decline in the use of Irinotecan versus oxaliplatin after Irinotecan became available as a generic in 2008.

"You get what you pay for," Peppercorn said. "If you incentivize doctors to give a more expensive drug instead of the generic, you're going to get use of more expensive drugs and higher spending. We need to deal with that and to figure out a way to pay for and support innovation and patient-centered, community-based cancer care."

But financial incentives are not the only factor guiding physician prescribing practices, a study of an Oncology Medical Home Pathway program found (Reinke, 2014). With this program, as billing was shifted largely from drug reimbursement codes to professional charges in order to reward oncologists for cognitive services they delivered, there was no significant difference in the delivery, type, or frequency of chemotherapy. There was neither more nor less use of generics over the time period of the study. "We need some caution in deciding that all of oncology practice is based solely on these financial incentives and will shift back and forth as we change the dollar signs," Peppercorn noted.

He added, however, that "no one is really standing up to defend 'buy and bill,' but instead arguing that the reimbursement laws we have right now provide inadequate support for care coordination and the complex disease management required for high-quality cancer care. You're giving something up if you don't pay for this care and ASP plus six is really inadequate to cover costs and the risk for purchasing and maintaining expensive cancer drugs."

Some practices need to keep $1 million worth of chemotherapy drugs in their inventory, he noted. "How can a small practice do that?" he asked. Large drug inventories are required because of the unpredictability of what patients will need. "Sometimes patients come in with a pain crisis that requires treatment that day. You can't wait a month in cancer care to adjust your supply to the demand," Peppercorn noted. Gould agreed that increasing drug prices "hits the cash flow of a practice very hard with more dollars tied up in inventory sitting on our shelves than we had in years past." He added that his practice's inventory used to amount to $500,000 worth of drug supplies, but now totals $1.2 million. "This ties up cash that can't be used for other operations," Gould said.

Peppercorn also gave a partial list of other services oncologists provide that he thought are not adequately reimbursed, including taking the

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time to adequately discuss the cost of care with patients, survivorship care planning, treatment navigation, palliative care, shared decision making, symptom management, and patient education. Administrative burden is also greater because of more burdensome precertification procedures, requirements for practices that participate in a CMS quality program, and a burgeoning of different guidelines for different payers to which physicians must adhere.

Gould added that declining reimbursement is a major problem for oncologists in private practice and that increasing costs of drugs and information technology (IT), administrative burden, and uncertainty are all conspiring to shift the site of cancer care from community practices to hospitals. Substantially contributing to the higher costs of running an oncology practice are the major investments physicians are currently making in electronic medical records (EMRs), he said. "These are not just boxes that sit on a desk, but they're complex IT systems that have to be maintained and can cost tens if not hundreds of [thousands of] dollars, so it's a very expensive endeavor," Gould noted.

Also challenging for private practices are the decreased rates of reimbursement for administration services, including fees for providing chemotherapy that were introduced by the Medicare Modernization Act of 2003. Administrative fees are currently being reimbursed at one-third the amount they were in 2003, according to Gould, and there has also been a two-thirds decline in the reimbursement for fees for providing chemotherapy. Sequestration introduced additional reduced reimbursement rates, not only because of the 2 percent reduced reimbursement rate for drugs, but a 2 percent reduced reimbursement rate for administration services, with practices offering radiology services receiving further cuts in their reimbursements. The CMS reimbursement rate for physicians administering cancer drugs has also decreased due to a new method the agency used to determine reimbursement for such services that was based on the Physician Practice Expense Survey done by the American Medical Association. In 2009, reimbursement for the first hour of chemotherapy administration, which is typically done by an oncologist, was reimbursed at $161, but this fell to about $147 in 2010, Hartstein reported. There also is a great deal of uncertainty about future reimbursement rates with the advent of the ACA, let alone the ups and downs of the general economy, Gould noted.

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340B

The 340B Drug Discount Program created in 1992 under the Public Health Service Act is administered by the Health Resources and Services Administration (HRSA). Several workshop speakers noted that it has substantially affected the market of cancer care and furthered consolidation of private practices and their migration to hospitals. Aimed at supporting practices that serve indigent patients eligible for Medicaid, 340B gives these practices discounts on drugs provided by a qualified entity, regardless of the insurance status of the patients to whom they are given. Conti noted that this program has expanded rapidly, with the number of participants doubling between 2001 and 2011 (see Figure 6). One-third of all hospitals participate in the program (Aitken, 2014).

Beginning in 2010, HRSA allowed qualified entities that do not have their own inhouse pharmacy to contract with freestanding retail pharmacies to provide drugs purchased at 340B prices to their communities. This prompted a large jump in the number of participating contract pharmacies. Such pharmacies now number more than 30,000, which is three times the number of participating entities (see Figure 7) (Avalere Health, 2013).

Although designed as a program that essentially transfers inexpensive drugs from pharmaceutical manufacturers to providers in exchange for providing charity care to the most needy U.S. populations, 340B-qualified providers can generate income by treating patients that have insurance paying reimbursement levels well above the discounted price at which these drugs are acquired, Conti stressed. She noted that Medicare reimbursements do not, by statute, reflect 340B discounts, both in terms of the amount and volume of the discount. "The opportunity to profit off the 340B has created an impetus for providers to push the envelope on the program's core intent. By opening outpatient clinics or pursuing affiliations for sites in affluent communities where patients will be well insured, this clearly causes mismatches between the prices of these therapies that the purchasers face and the reimbursement price they receive once they use them in the patient. So it creates some incentives for revenue seeking," she said.

Conti noted that disproportionate share hospitals (DSHs) are the most prominent type of hospital that qualifies for the 340B based on the vulnerability of their inpatient census, but their outpatient census is not factored into this assessment. A study done by Conti and Peter Bach, director of the Center for Health Policy and Outcomes at Memorial Sloan Kettering, found that although DSH institutions serve populations that are relatively less well-off compared to the overall U.S. population, their affiliated clinics

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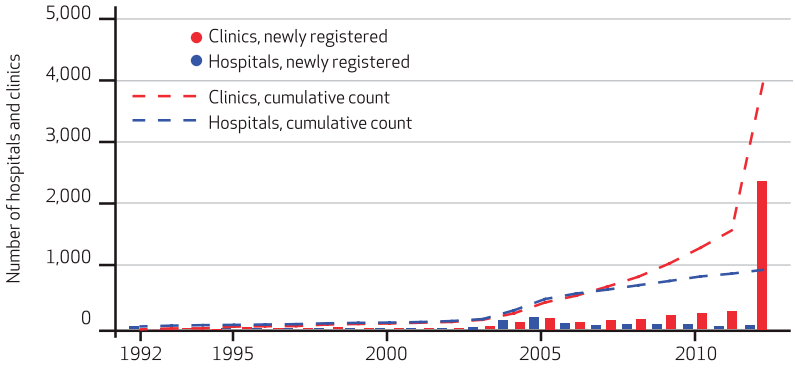

FIGURE 6 Numbers of disproportionate share hospitals and their affiliated outpatient clinics in the 340B program, 1992-2012.
SOURCES: Conti presentation, June 9, 2014; Conti and Bach, 2014.

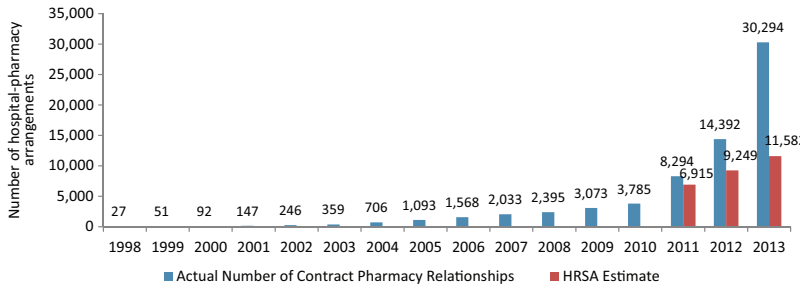

FIGURE 7 Contract pharmacy relationships are growing. Each relationship between a 340B entity and a contract pharmacy is counted separately for this analysis. Some pharmacies have relationships with more than one 340B entity; thus, those pharmacies are counted more than once in this analysis.
NOTE: HRSA = Health Resources and Services Administration.
SOURCES: Conti presentation, June 9, 2014; Avalere Analysis of HRSA Enrollment Data as of November 5, 2013.

largely serve equivalent or somewhat more affluent populations than that of patients seen in DSH hospitals or that of the U.S. population as a whole (see Figure 8). Conti remarked, "These affiliated clinics appear to be serving increasingly wealthy and less indigent patient populations over time, a trend that accelerated after around 2008. Based on this result, we suggest that the program core goals have likely been eroded by these program expansions into the recent clinic affiliates."

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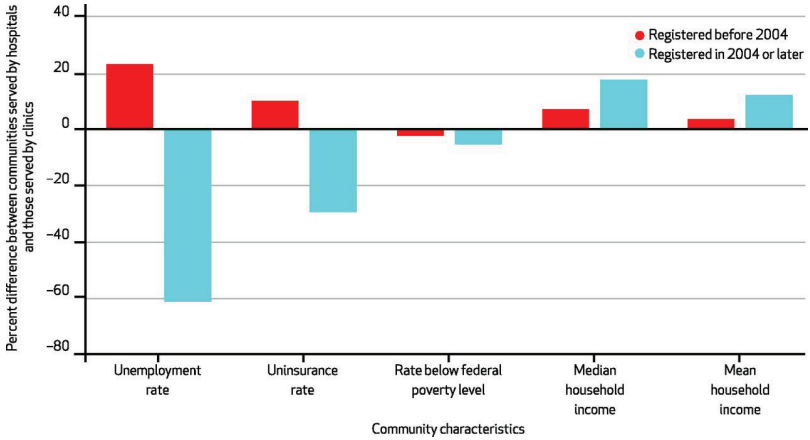

FIGURE 8 Socioeconomic characteristics of communities served by hospital-affiliated clinics in comparison to characteristics of communities served by disproportionate share hospitals, by time of registration for the 340B program.
SOURCES: Conti presentation, June 9, 2014; Conti and Bach. 2014.

Another study she did demonstrated that compared to all prescriptions dispensed through the Walgreens setting in 2012, those made by 340B-qualified entities were less likely to be for generic drugs (Clark et al., in press). In addition, the 340B-qualified entities prescribed these drugs to individuals that, although they met charity care provisions, also most commonly were covered under commercial insurers or under Medicare. "Therefore the opportunity to actually generate revenue through this Walgreens program exists," Conti noted.

"Our findings suggest that gaining access to the 340B discounts may be one important rationale motivating merger and acquisition activity" of practices, Conti concluded. She suggested this fostered both physician mergers and affiliations amongst themselves, as well as physician practice affiliations with hospitals and hospital mergers, all of which have substantially increased since 2010, when HRSA began allowing subcontracting with commercial pharmacies.

Conti speculated that when drug manufacturers set their launch price of new drugs, they are likely considering the increasing availability of 340B discounts and pricing their products higher to compensate. She concluded that the benefit of the 340B program depends on the dedication of qualifying institutions to treat the most vulnerable populations, and suggested

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this be monitored by increasing transparency in the program. She also questioned whether the benefits of the program are worth the costs of physician practice and hospital consolidations, and what the costs are to the individual patient, insurer, and employer because these payers do not provide reimbursements that match those of the 340B program.

Consolidation of Practices

Gould asserted that increased drug and administrative costs, combined with decreased reimbursements and the 340B program, have led to a situation in which many oncology private practices cannot compete with hospitals. Consequently, more practices have merged with each other, aligned with hospitals, or closed, according to Gould (see Figure 9). This consolidation of practices reduces competition that normally keeps costs for care in check. "When we have consolidated providers, they have market power and are able to name their price and you have to pay it," Conti noted. Hartstein added, "The changes in CMS payments have shifted the incentives and many physician practices have consequently become hospital outpatient departments."

The most common current trend is for private practices to align with hospitals because it offers the most benefits for both physicians and hospitals, but not necessarily for patients, Gould said. Aligning with hospitals relieves physicians of the financial and administrative burdens of a private practice, and enables them to spend more time on patient care, according to Gould. Hospitals benefit from such alignments by reducing their competition and having an array of key specialists in an integrated network that can be leveraged for payer contracting and can enable them to participate in the 340B program. The ACA's emphasis on bundling reimbursement models and comprehensive coordinated care is also fostering hospitals to become bigger and purchase private practices, Hammelman pointed out. He added, "There are a lot of physician offices out there that can't afford a lot of the drugs they need—that don't have the capital you'd need to purchase these drugs and stay afloat. So they look for a hospital partner," Hammelman said.

No studies have documented a difference in quality of cancer care given in hospitals versus given in private practices, according to Gould, but he raised questions about the care patients receive from a hospital-based organization, which is more bureaucratic in nature and may be less focused on oncology services compared to a small private practice that makes cancer

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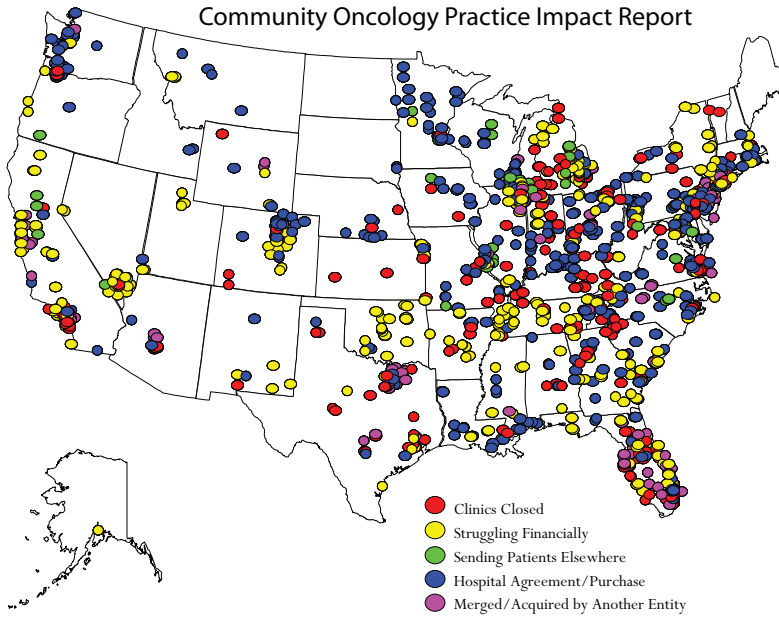

FIGURE 9 Cancer clinic realignment. Many U.S. community oncology clinics have closed, report that they are struggling financially, are sending patients elsewhere, have a hospital agreement, or have merged or been acquired by another entity.
SOURCE: Gould presentation, June 9, 2014.

care their sole focus. "I suspect not only is the cost of care going to be lower in the physician practice setting, but the quality of care will be better as well," Gould said. Peppercorn added that if hospital care is similar to care received at an academic health center, "Although you're pouring more resources into the system, the oncologist has less and less time to spend with the patient."

POTENTIAL SOLUTIONS

Many workshop participants argued that current practices are not sustainable; there is a need to recognize the finite health care resources available and set priorities and incentives accordingly. The many stakeholders in cancer care all have a role to play in addressing the challenge, including patients and their care providers, health care payers, and the pharmaceutical

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industry. Chalkidou pointed out that establishing those priorities requires political, professional, and general public backing so that trade-offs are managed appropriately, and ensuring patient access to effective cancer drugs. Whether this can occur in a country such as the United States is questionable, she noted, and asked whether the expanded coverage with the ACA might drive some form of open priority setting as more people gain access to the care they need. Chalkidou also described how the United Kingdom sets it health care priorities, as summarized in Box 3.

Fendrick stressed that "More care is often not better care. As someone who has been challenged on the floor of Capitol Hill as a rationer, my response is that I ration harmful care." He suggested eliminating care for which the evidence convincingly shows a lack of value or potential harm to

BOX 3
Value-Based Assessments in the United Kingdom

Kalipso Chalkidou reported that Great Britain's National Health Service (NHS) has a constitution that ensures patients have the right to access drugs and treatments that have been recommended by the National Institute for Health Care Excellence (NICE) for use in the NHS, if their doctors determine they are clinically appropriate. Patients also have the right to expect local decisions on funding of other drugs and treatments to be made rationally following a proper consideration of the evidence. If the local NHS decides not to fund a drug or treatment that a patient and his or her doctor believes is appropriate, they must explain that decision to the patient.

To help make their recommendations, NICE has recently developed methodology for determining "value-based assessments," which can be used to inform value-based pricing. These assessments are based on two attributes. One is the burden of illness or severity of the disease and how much it shortens quality-adjusted life years. The other attribute is the wider societal benefit of the treatment, which is a proxy for productivity. The end result of the mathematical formula used in this assessment is that priority is given to drugs that address very severe conditions in people near the end of their lives, and also to drugs that address conditions that mostly affect younger people. "The idea is to both rate highly drugs that attack conditions that kill you, and drugs that attack the conditions that affect the very young," Chalkidou said.

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individuals. "The path to avoid that care is the path to better health and we are increasingly being put in charge of being the stewards of the collected good of a scarce resource," Fendrick added.

A few participants mentioned the Choosing Wisely program, through which professional societies have provided lists of oncology treatments of little to no value. Such a program, which has been led by the American Board of Internal Medicine, may constrain prices indirectly by encouraging and providing information for value-based use. More than 60 medical specialties are participating in Choosing Wisely campaigns.

Nasso stressed, "We must collectively change the discussion on health care costs from 'how much' to 'how well' because we have enough money in the system. It is just that some things we are buying too much of, while others we are buying too little of." She noted that experts have estimated each year that tens if not hundreds of billion dollars per year are wasted on health care services that either do not help patients or actually harm them (IOM, 2013a).

Several participants emphasized the need to make the cancer care market more competitive. Patricia Danzon, Celia Moh professor at the Wharton School of the University of Pennsylvania, noted that other countries have reached the conclusion that if patients have comprehensive insurance coverage, then some constraint on price and/or reimbursement is necessary to reign in prices and expenditures because patients will be price insensitive. "In that environment, manufacturers have incentives to charge very high prices and we cannot blame them. They respond to the system we create," she said. Danzon pointed out that most developed countries have or are moving toward systems that require evidence of effectiveness, usually comparative effectiveness, as part of the negotiation of price and reimbursement at drug launch.

But Hartstein noted that CMS, which is the biggest payer for cancer care in this country, is prohibited from negotiating prices and interfering with the practice of medicine. In addition, the ACA specifically prohibits the use of quality-adjusted life years (QALYs) or incremental cost effectiveness ratios (ICERs) or other metrics for the survival benefit of a treatment from inclusion in some of the new pilot programs it funds. Howell said, "The challenge is how do we bring in the free market system and not create this law of unintended consequence, which feels like where we are today. We didn't intend to shift oncology to higher cost-of-care settings, but it has clearly happened, as the data show. So how do we balance that?" Chalkidou pointed out the irony that the United States supports the free market and

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competition "and here we have a pharmaceutical market that's far from being a functional market." She suggested putting more information in the public domain to inform payment decisions.

Fortner raised the question of whether "me-too drugs" that mimic innovative drugs might forestall drug shortages and encourage competition. Bach responded that even if a drug has the same route of administration, indications, and mechanism of action, and in many cases grows out of the same platform for drug development, it is treated as unique by the FDA, which enables it to have its own pricing code if it is a physician-administered drug. Consequently, "when a me-too comes along, it's not classified as a me-too because the entire regulatory apparatus classifies essentially every new drug for cancer as its own special thing," Bach said. So even though the first drug on which the me-too drug is modeled invested much more in research and development than the me-too drug that follows it, the latter drug can be irrationally priced more, he added.

Zafar responded, "Data presented at ASCO [American Society of Clinical Oncology] this year showed that the single greatest predictor of price of a follow-up drug is not effectiveness and toxicity, but the price of the drug that came before it" (ASCO, 2014). Howell stressed, "We need systems that allow us to be able to have products compete on value."

Newcomer suggested that instead of providing pharmaceutical firms with patent protection for long periods of time, give them a guaranteed price on the market. This is likely to stimulate competition by encouraging either more me-too drugs that could be priced cheaper, or production of generics. But Bastian responded that rather than tampering with the American patent system, which is seen as a mainstay for innovation, it would be better to address the pricing issue by other levers within the system, including value assessments. He cautioned that in the developing world when patents were violated, it destroyed the architecture and investment case for these therapy areas.

Some workshop participants suggested more specific potential solutions to improve patient access to cancer care and to lower the costs of that care. These solutions involved changing incentives for patients, physicians, or drug manufacturers.

Patient Incentives

A number of participants noted the lack of incentives for well-insured patients to opt for lower cost cancer care, given that they often have to

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invest little of their own money in such care. Hammelman noted that after his father signed up for Medicare, he and his physician became insensitive to the cost of his cancer therapy and his father proceeded to go on a very high-cost chemotherapy for the next 2 years. "He kept saying to his medical oncologist, 'What's going on here?' and the oncologist said, 'As long as Medicare keeps paying, we'll just keep doing it,'" Hammelman said. Fendrick added, "The evidence is overwhelming in cancer that the consumer doesn't care about overall costs, so we should start aligning the consumer incentive around what they are paying to what they are buying." Many workshop participants suggested that value-based insurance designs and frank conversations with patients about the costs and benefits of their treatment options might help lower the excessive costs of cancer care.

Traditionally, insurance companies incentivize patients and physicians to choose a lower cost but equally effective drug by establishing tiered cost-sharing rates, in which patients have a smaller co-pay for lower cost drugs than for more expensive drugs. Although this has been effective for relatively low-cost drugs such as statins or antiulcer medicines, Danzon noted, it hasn't worked for most cancer therapies because even for patients with good insurance coverage, "one course of treatment blows through the upper limit on cost sharing so manufacturers have realized that there really is nothing that is constraining prices," she said. On the other hand, it was also noted that for many patients, the cost-sharing limit is still relatively high compared to their income and assets.

Fendrick suggested that cost sharing with patients could be more effective than what is currently done. He noted that because of the way most health insurance plans are currently set up, most Americans pay the same cost share for every type of doctor within their network, every diagnostic test, and every drug within the tier of the formulary. This means that they will pay, for example, the same 20 percent for drugs that cure a particular type of cancer 90 percent of the time as well as for drugs that never cure cancer. "This one-size-fits-all benefit design fails to acknowledge the differences in clinical value among medical interventions and makes no sense," he said.

He noted that cost sharing can "make patients think twice about paying for health care they don't need. In some situations . . . , not only are they harmed, it actually costs all of us more." However, he also noted that studies show that financial burden is one of the many reasons why people do not adhere to treatment regimens, and that rising co-payments may worsen health disparities and adversely affect health, particularly among patients living in low-income areas (Chernew et al., 2008).

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Value-Based Insurance Design

Fendrick suggested that insurance plans should incentivize patients to choose high-value treatment options by setting the consumer cost-sharing level on clinical benefit, not acquisition price, of the service. Studies show such value-based insurance designs improve patient adherence to treatment and lower their costs without a significant difference in total spending (Lee et al., 2013). One study found that full drug coverage for heart disease care in non-white patients increased adherence as well as reduced total health care spending by 70 percent in that group, "suggesting that targeted value-based insurance design programs not only improve outcomes, but save money and reduce disparities," Fendrick stressed (Choudhry et al., 2014).

According to Fendrick, over the past 10 years, nearly a thousand private and public insurance programs have implemented such designs to a limited degree. Some do it for certain classes of drugs, or for a few high-value diagnostic tests or treatments, such as diabetic eye exams, and physical therapy after hip replacement. "They are starting to understand that certain services are high value for which they should not create a substantial financial burden for individual clients and patients to get them—that it's a good idea for individuals to pay less for statins than they do for heartburn medicine, or to pay less for insulin than for a drug that makes toenail fungus go away or hair grow back," Fendrick said.

He said value-based insurance design has bipartisan support from multiple stakeholders, and was included in the ACA, which eliminates co-pays for primary preventive services given a high ranking by the U.S. Preventive Services Task Force, HRSA, and the Advisory Committee on Immunization Practices at the Centers for Disease Control and Prevention (CDC). A provision of this law prohibits cost sharing for more than 60 evidence-based preventive services, which has expanded coverage to approximately 105 million Americans, Fendrick said.

But he noted the challenge in oncology is that "even if you pick a service to be high or low value, that service depends on who gets it, who provides it, and where." He concluded his presentation by suggesting value-based insurance designs be applied to oncology by imposing no more than modest cost sharing to high-value services, and by reducing cost sharing in accordance with patient- or disease-specific characteristics. "This idea of making sure people have a lower cost share if they test positive for a marker that indicates a higher likelihood of a medication success is a no brainer and something that both providers and plans can implement," Fendrick said. He

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also suggested relieving patients from cost sharing after they have failed a lower cost medication, which Fendrick called "rewarding the good soldier" because it provides some type of cost-share reduction if patients have tried less expensive medications first and found them to be ineffective or too toxic. In addition, he suggested using cost sharing to encourage patients to select high-performing providers and settings. "Value-based insurance design implemented even in a baby-step way should be part of the solution to enhancing efficiency in cancer care, and such cost containment efforts should not produce preventable reductions in quality of care," Fendrick concluded.

But Kevin Olson, executive medical director at Providence Cancer Center, noted that "consensus on value definition is lacking," especially when it comes to making value assessments of end-of-life cancer care. These assessments can be especially thorny because of the strong emotions and ethical issues attached to them, noted Olson. When the state of Oregon was trying to make those assessments for its Medicaid program, "We heard from advocacy groups who said there should be no role for the state or health care plans to define what should be done near the end of life. Their position was that it should be between the doctor and the patient," he said. The doctors he polled, in contrast, expressed relief that they had some administrative support for telling patients when the costs and risks of treatment far outweighed the benefits, because patients often find it difficult to reconcile themselves to the concept of forgoing disease-targeted chemotherapy. Palliative care to address symptoms was always covered, whether or not the patient's illness was considered terminal.

Cost Discussions with Patients

Several participants suggested physicians engage in discussions with their patients about the costs of their care when considering a treatment plan (IOM, 2013c). A study by Zafar found that half of patients expressed some desire to talk to their oncologist about the cost of their treatment, but only 19 percent had that cost discussion. Among those that had the discussion, more than half said it decreased their out-of-pocket expenses. "These conversations can be done without knowing a lot of details of drug cost or insurance plans, and is really important in cancer where most of the time we don't really have a lot of alternatives," he said. "Until we can get prices under control, encouraging communication around cost is a first and fairly easy step."

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Zafar noted that there are websites and apps that can illustrate the costs and benefits of different types of care to support these discussions. He is currently developing a Web-based interactive app that directs patients to financial resources specific to their needs, and encourages them to talk to their doctors about the financial burden of their care. "This app can support patients and doctors to be on the same page about this," he said. Fendrick suggested EMRs be designed to make it easier to show the patient what the evidence base for a treatment is and how much it will cost. He advocated for making sure health consumers' incentives are aligned with the incentives for other stakeholders in an effort to constrain the costs of cancer care.

Nasso said that although many patients want to talk about the cost of their cancer care, not all do. "It is important to ask the patient if that is what they want to talk about. It should be their prerogative if they don't want to have that conversation," she stressed. Nasso also cautioned against the assumption that patients can solve the massive problem of excessive costs in cancer care by the treatment decisions they make. She said it was too much of a burden to put on patients to expect them to advocate for lower costs. Instead she suggested that patient advocates not currently undergoing cancer care take more of a role in advocating for lower cost drugs and other cancer therapies.

Nasso also stressed that physicians can have honest discussions with their patients about the value of their care options—to communicate the evidence so they understand, for example, that "response" does not mean they are going to be cured. "We want to make sure we can give them hope, but make sure they also understand really what the possibilities are, the benefits and risks of every treatment and the financial costs are a part of that, if that is what they want to discuss," she said.

Zafar agreed with the importance of having a conversation with patients, not just about the costs, but about the value of the cancer care options available to them. "I bet if you talked to my patients immediately after they walked out of the room, they probably wouldn't have a very good idea of exactly how much life expectancy or quality of life they're going to get out of this very expensive treatment that I'm giving them. If patients were informed consistently of the possible outcomes and risks, they might make different choices." He noted a study he is currently conducting in which patients have declined certain treatments once they understood the relative value of those treatments, including a patient with advanced, incurable pancreatic cancer who recognized that he was underinsured and that

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his family would end up with thousands of dollars in medical bills after he died if he opted to receive an expensive therapy.

Conti added that when it comes to life-threatening disease, people are willing to pay for a cure. Her study found that the largest predictor of diffusion of cancer drugs was the underlying mortality of the cancer itself. Bastian said another study found that patients are willing to pay for just the hope that their cancer will be cured (Lakdawalla et al., 2012).

Zafar noted that physicians have difficulty discussing the cost of care with their patients, but that it is in the best interest of their patients to do so, given that some studies find as many as one-third of patients struggle with their medical bills. "We care deeply about our patients and want to make sure we're giving them appropriate care, but many of us are unaware of what our patients are struggling with [financially]," he said.

But others thought patients struggling for their life and debilitated by their cancer or its treatment should not have to worry about the cost of care, and that doctors should bear the burden of determining the most cost-effective treatment option for their patients. "Let's have patients be as immune to this as they should be. They're going through cancer care," Hammelman stressed. Bastian noted a study that found that although the cost of their care is one of cancer patients' top four concerns, quality-of-life concerns about the effect of the cancer on their lives or their families, as well as concerns about dying from the cancer, take precedence (Ramers-Verhoeven et al., 2013).

The Cost of Convenience

Some discussion centered around the newer oral cancer therapies and whether the convenience of these drugs to the patient is worth their high costs. In some cases, such as multiple myeloma, taking oral anticancer medications is not a matter of convenience because there are no IV equivalents for the oral drugs available. However, for cases in which treatment options include both oral and IV drugs, convenience may be a factor in treatment selection.

Nasso pointed out that "When you don't feel well and going to the doctor is a big ordeal, especially if you have to travel a long distance, convenience can be really important." But the value of that convenience may not be the same for every patient, she added, noting that it may be more important to a patient who is working versus one who is not, for example. Some patients who live near a cancer treatment facility may find it less oner-

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ous to go for an intravenous treatment, so a more expensive oral treatment would not have that much value for them. "It's not a one-size-fits-all issue so you should have that discussion with the patient and take into account what the patient really values," she said.

But Peppercorn noted that the difference between taking 5FU and leucovorin by infusion, or just taking 5FU orally alone, is $25,000. "Why would anyone want to pick the oral 5FU? Is that convenience really worth around $25,000? Is that a decision we should let a patient make if they don't have any vested interest in the cost?" he asked.

Chalkidou responded that in the UK health care system, any time a new expensive cancer treatment is approved, it limits the resources available for other, perhaps more valuable, treatments, such as dialysis. "That's the bigger question we're trying to address that you don't get at the individual patient-physician interchange. I don't think it's a minor inconvenience in order to have enough money to pay for other therapies, which may have more benefit down the line for other cancer patients," she said.

Peppercorn pointed out that "$25,000 for convenience when median household income is $40,000 is just too high. Innovation is not just about improving survival, but about convenience … and quality of life. However, we need to realize that this high price of innovation wouldn't be priced that way if the patients saw the costs," he said. Scott Ramsey, full member, Fred Hutchinson Cancer Research Center, and director, Hutchinson Institute for Cancer Outcomes Research, added that the greater lack of patient adherence linked to some oral medications makes their value even worse. "I'm not sure this argument about convenience works if we're looking at it in terms of efficacy," he said. Peppercorn added that there is less dose adjustment flexibility in response to the toxicity that develops with oral medications, which typically only come in one or two dosage forms. Consequently a lot of oral drugs are wasted because of switching to lower doses after the higher dose has been purchased, and the perfect oral dose is difficult to accurately achieve.

Drugs given orally, as opposed to intravenously, are not usually fully reimbursable by insurers and require a co-payment that can be a fixed amount or a percentage of the total cost, Peppercorn said. Many of the newer oral cancer drugs are quite expensive, which causes out-of-pocket costs to be in the thousands of dollars for many patients. Recognizing this, 34 states and the District of Columbia have passed oral parity laws with the requirement that the reimbursement benefit for oral chemotherapy drugs cannot be any lower than the benefit for intravenously administered cancer

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therapy drugs. Peppercorn stressed that Medicare is excluded from oral parity laws. However, federal legislation was introduced in both the House and Senate in 2013.

Jeffery Ward, an oncologist at the Swedish Medical Center, noted that the payment scheme ASCO has proposed makes payment for the management of the patients the same whether they are on oral drugs or on drugs given intravenously. A goal is to add a management fee into monthly bundled payments as an alternative for traditional "buy and bill" reimbursement schemes. But Ward also noted that ASCO's payment strategy does not provide adequate incentives for physicians to consider the cost for the medicines they provide.

Physician Incentives

Several workshop participants suggested physician incentives for reducing the costs of cancer care, including incentives for prescribing low-cost drugs; episode-based reimbursement to adequately reimburse all the services oncologists provide for their patients while containing costs by reducing hospitalizations and other high-cost care; and policies to make private oncology practices more competitive with hospital-based practices.

Incentives for Prescribing Low-Cost Drugs

Recognizing that physicians substantially affect the cost of cancer care by their prescribing practices, several workshop participants made suggestions for how to prompt physicians to prescribe cost-effective treatments and to choose lower cost treatments if they are as effective as higher priced options. Zafar suggested educating physicians on cost-cutting strategies, such as prescribing generics, buying medicines in bulk, offering less expensive therapeutic alternatives, reviewing medication lists to eliminate medicines no longer needed by the patient, discount cards, and splitting pills prescribed at a higher dose (Arora et al., 2013).

Gould suggested greater use of physician practice guidelines and more consistency among payers as to which guidelines they use. "Our practice administrator jokes that when the patients come in they're going to be given a hat with what payer they have so we can use the right guidelines for them," he said. He added that the approach the Community Oncology Alliance takes is to view the total care of the patient, not just the drugs they are prescribed. That total care includes providing care in the lowest cost

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setting, which is the physician's office, as well as trying to limit the use of high-cost resources, such as hospitals and chemotherapy at the end of life. "I ultimately see the medical practice evolving to the oncology medical home model where the physician takes responsibility for the total care of the patient, and being more thoughtful in the use of our limited resources," Gould said. Zafar added, "We need to bring cost and value to the forefront. Every discussant of every major research project presented at ASCO should be asked to talk about value."

Bastian noted that physicians will have to prescribe the generic equivalent of biologic drugs, known as biosimilars, in order for the cost of cancer drugs to significantly drop, given the large cancer market share of these drugs. In Europe the costs of such biosimilars are 20 to 30 percent lower than the original biologics they are based on, but regulatory confusion has hampered greater use of biosimilars in the United States, he said. Because the FDA has not yet determined what standards will be required to meet the threshold of "interchangeability" for biosimilars, most states have not developed formal laws regarding their use.

Bastian noted that such laws are an opportunity to significantly lower cancer drugs costs. "If you could reduce the price of 50 percent of the market share by 20 to 30 percent, I don't think we'd even be having this discussion right now," he said. But he noted that physicians may be fearful of lawsuits over prescribing biosimilars if they are not similar enough to biologics. Peppercorn added that due to their complexity, the cost and time involved in making a biosimilar may be as great as bringing a new drug to market. So it is unclear if there will be a cost saving reaped by having biosimilars on the market, he said.

Peppercorn also called for reimbursement reform so generics are reimbursed at a greater rate that will incentivize more physicians to prescribe them, and more manufacturers to produce them. He noted that Ezekiel Emanuel has suggested a reimbursement rate of ASP plus 30 percent for generics (Emanuel, 2011).

But drug-prescribing practices are just one part of the high costs of cancer care that needs to be addressed by policy measures, several participants noted. Peppercorn suggested oncologists be adequately compensated for the complex and time-consuming care they offer patients that goes beyond the therapies they prescribe. In particular, he suggested that the chemotherapy administration fee be reimbursed separately from the drug fee. "We could pay more for the chemo administration fee, which is independent from the price of the drug. Why should they be lumped together?" he said.

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Patricia Ganz, director of cancer prevention and control research at the University of California, Los Angeles, Jonsson Comprehensive Cancer Center, added that the downstream costs related to addressing adverse side effects from chemotherapies substantially increase the cost of cancer care. She suggested focusing on those issues in addition to the costs of the drugs or their administration. Newcomer concurred and stressed that 65 to 75 percent of cancer care costs are for something other than drugs. "Paying attention to the tests we order, the side effects we create, and the symptoms we manage, and dealing with these early to keep people out of the hospital, is to me the far greater way of saving money for cancer care. And the patient is going to benefit if we deal with these issues faster and more appropriately so they suffer less," he said.

Some participants called for greater use of specialty pharmacies that offer supportive care to patients undergoing chemotherapy. These pharmacies distribute highly expensive drugs that require additional management and sometimes sophisticated handling. They often offer 24-hours-a-day phone counseling with an expert pharmacist. Such counseling helps patients manage their side effects and ensures they are getting the maximum benefit from their therapy, Newcomer reported. A study by UnitedHealthcare found that the creation of their own specialty pharmacy for their members reduced the cost of their care by about $13,000 per patient, mainly due to better management of toxicity, fewer hospitalizations, and less time spent in the hospital, Newcomer noted (Tschida, 2012). He suggested greater use of specialty pharmacies to not only help reduce the costs of cancer care, but to help patients manage their medicines well. Kolodziej added that the National Comprehensive Cancer Network recommends that specialty pharmacies counsel patients receiving oral chemotherapy to improve treatment adherence.

Peppercorn stressed replacing the traditional buy and bill way of doing business in oncology. "It's irrational and unsustainable and does not adequately compensate those other important aspects of care," he said. Nasso emphasized that reducing the cost of cancer drugs will be just part of the solution, and she agreed that the supportive care physicians provide to their cancer patients should be reimbursed at a higher rate. "We need to look at the whole system and how it is incentivized and whether we are paying for the care that patients really value, which is the time they spend with their physician and their health care team to get the care that they need," she said. That care includes the time spent making sure patients understand their treatment options and palliative care to manage symptoms and side effects and to prevent hospitalization, she noted.

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Episode-Based Reimbursement

Some participants at the workshop suggested pay-for-performance or bundled payments as models for reimbursing oncologists for all the care they provide to their patients. Thomas Feeley, Helen Shafer Fly distinguished professor of Anesthesiology, Division of Anesthesiology and Critical Care, The University of Texas MD Anderson Cancer Center (MDACC), noted that the fee-for-service model currently used has been blamed for our current national health care crisis, and that with a bundled episode-based payment scheme, the hope is that instead of paying for the volume of care provided, the actual costs of the care needed are reimbursed. This should incentivize efficient, higher quality care while reducing the administrative burden of processing claims, he said. He argued that an episode-based payment scheme is also likely to be a better model than patient capitation models used previously, in which physicians were paid a set amount per patient, regardless of the care they received.

Peppercorn suggested Medicare lead the way in reimbursement reform because it dominates the market for cancer care and other insurers tend to follow the practices of Medicare. Zafar concurred, adding that efforts to contain costs made by private payers, hospital, doctors, and other health care providers "won't make a lick of difference if we don't have Medicare involved in this as well." But Hartstein cautioned that Medicare bases its reimbursement rates on data for a population 65 years of age or older and those that are disabled. He said it concerns him that other insurers use these rates when they may not be applicable to the types of patients they insure. "Establish payment rates based on your own data and patient populations," he stressed.

Hartstein noted that the ACA created the Bundled Care for Payments Improvement Initiative, which includes four innovative new payment models. Under this initiative, organizations will enter into payment arrangements that include financial and performance accountability for episodes of care (CMS, 2014). Lump sum payments are given for these episodes of care rather than the separate payments Medicare traditionally gives to providers for each service they perform for beneficiaries during a single illness or course of treatment. He said this traditional approach can result in fragmented care with minimal coordination across providers and health care settings. It also rewards the quantity of services offered by providers rather than the quality of care furnished. Over the course of the 3-year Bundled Care for Payments Improvement Initiative, which began in 2013, CMS will

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be working with participating organizations to assess whether the models being tested result in improved patient care and lower costs to Medicare.

Feeley noted that Medicare recently began bundling payments for dialysis, so instead of making separate payments for each of the drugs administered to patients undergoing dialysis, dialysis facilities were given a single bundled payment for each dialysis episode. At the same time, Medicare required quality measures, such as blood sugar indicators, to ensure this payment system did not compromise the care patients received. The use of drugs for dialysis was reduced by 10 to 30 percent when the bundled payment system was used, Feeley reported. But Conti cautioned against generalizing this example to oncology because "we don't have great quality metrics for much of the outcomes related to cancer care, so we're stuck with process measures, which may or may not be correlated with outcomes. The opportunity for gaming and also for stinting looms its head in those cases."

Feeley noted that a bundled payment model designed specifically for cancer care was not addressed in the ACA, and an IOM report found no current evidence for the effectiveness of such a model in controlling costs of cancer treatment (IOM, 2013c). But CMS and its Center for Medicare & Medicaid Innovation have indicated their interest in new cancer reimbursement models in 2014. In addition, the Center for American Progress initiated an effort to develop models of episode-based payments for lung and colon cancer in September 2013, and the MDACC just embarked on a 4-year project to model a bundled pricing program. At least one major cancer provider network in the Northeast is also developing a bundling payment model with a private insurer, and the American Medical Association and ASCO have proposals for bundling payments, Feeley said.

The MDACC developed its own episode-based pricing model for head and neck cancer with the aims of reflecting the true cost of care delivery, capturing the entire multidisciplinary care experience, and tying care to measurable outcomes meaningful to both patients and physicians. The Center identified 160 processes related to head and neck cancer that were incorporated into its model, along with considerations of financial risks and margin of profit that it is using to guide the episode-based pricing being provided by a private insurer starting in September 2014, Feeley reported. During this process, the MDACC discovered that the biggest differentiator for cost of treatment was not the stage and type of cancer, but the treatment doctors prescribed.

Eight different bundled payment categories are part of the model, with bundles based on type of treatment and the comorbidities of the patient

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treated. The bundled payment covers all treatment-related costs occurring within a 1-year period for newly diagnosed patients, including such factors as radiation therapy work-up and the costs of participating in clinical trials. "We will deliver all of the services for a predetermined price, regardless of the services that are utilized," Feeley said. Patients in the bundling pilot will have the same co-payment structure as they previously had, he added. The outcome measures used for the pilot include those related to survival, quality of life, readmissions, and return to work.

Incentives for Oncology Private Practices

Noting that the cost of cancer care is greater when it occurs in the hospital as opposed to a private practice, some participants suggested legislation or policies to incentivize oncologists to stay in private practice or to make their practices more competitive than those offered by a hospital. This could include payment parity between hospitals and the outpatient setting, but others noted the potential inadequacy of such actions given the additional costs hospitals may have due to providing emergency room care to uninsured patients. Gould reported that Congress is currently considering several initiatives in this regard, including House Bill 1416, which would remove the sequestration cuts to Medicare Part B drugs, and House Bill 800, which would remove prompt pay discounts from the calculation of the ASP. This prompt pay discount is given to the distributors, not the oncologist, who pays his or her bill promptly, and ultimately reduces the drug reimbursement rate to the medical oncologist. In addition, House Bill 2869 seeks to have parity in payment for administrative services for the hospital versus the physician office.

The Community Oncology Alliance supports all these initiatives, Gould noted. He added, "This is a ripe area for helping control costs and relieving some of the financial burden on patients since [the cost of] care received in the hospital outpatient setting is significantly higher than that in the physician office. For a very small investment, community oncology can be preserved and again help provide expert care close to the patient's home."

Hartstein reported that the Medicare Payment Advisory Committee (MedPAC) has recommended that Medicare pay evaluation and management services at the same rate regardless of whether such patient visit services are provided in a hospital outpatient department or in an off-campus provider-based department, which is akin to a physician office. CMS is currently gathering data on how many services are provided in these settings

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and other information that will guide its reimbursement practices in this regard, according to Hartstein.

Conti added that HRSA is expected to come out with a new regulation that will better define who can qualify for the 340B program, as well as an information system that can track whether patients are receiving Medicaid rebates for the same drugs, so as to avoid duplicate discounts.

Drug Manufacturer Incentives

Addressing Drug Shortages

Peppercorn suggested that given the current drug shortages due to a lack of financial incentives to produce generic drugs, the federal government could consider developing them. He also suggested reimbursing less for new drugs, as many countries in Europe do already. Zafar suggested accelerating or prioritizing regulatory support for low-cost cancer drugs or those with supply issues. He noted that a few private payers have changed their reimbursement rate so it is higher for generic or low-cost products. He suggested something similar could be done to incentivize people to use biosimilars. Zafar also suggested that insurers be more sensitive to price changes in chemotherapies and adjust their reimbursement levels more quickly than they have in the past to help stabilize the supply and demand for cancer drugs.

Value-Based Pricing

Several workshop participants suggested strategies to counter the escalating costs of cancer drugs, including reimbursement strategies such as value-based pricing. Danzon described valued based pricing, in which higher prices are paid for drugs showing better treatment outcomes, to discourage the use of ineffective yet costly therapies (see Box 4). She stressed that value means not just outcome, but outcome per dollar spent (Danzon et al., 2013). "Economists care about value because if we were to allocate our resources based on value, we would achieve the maximum health gain for the budget we spend and equity between different patient groups and classes of care." She noted such pricing would entail an independent agency that would evaluate the evidence for a new drug relative to comparators. Each payer would determine its threshold willingness to pay for either the particular type of drug, drugs in the same disease category, or drugs in

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BOX 4
Overview of Value-Based Pricing

- Outcomes assessment: An independent agency (or individual payer) evaluates outcomes evidence for new drugs vs. comparators
- Each payer sets its value threshold (willingness to pay) required for reimbursement, e.g., $100,000 per quality-adjusted life year (could differ by indication)
- Based on its budget, enrollees' income, preferences, etc.
- Manufacturers set the price, given their drug's outcomes evidence and the payer's value threshold required for reimbursement
- Payers reimburse for patients if the drug meets the expected value threshold
- Co-payments are modest, to ensure affordability for patients

SOURCES: Danzon presentation; Danzon et al., 2013.

general. This threshold value could be expressed as a dollar amount per life year saved and could differ across payers and by indication.

The manufacturer could freely set its drug price, but subject to meeting that value threshold. Payers would pay for drugs that meet the value threshold and co-payments would be kept at a modest level so that cost sharing would not be a barrier to access for patients for whom the drug is of proven value. If prelaunch outcomes evidence is limited, there could be provisional reimbursement, with postlaunch data collection and price adjustment if needed.

Kolodziej concurred that value-based pricing for cancer drugs was a useful approach, saying "You get what you pay for—if it works, you get paid a little bit more, and if it doesn't work, you don't get paid so much." He pointed out that a lot of money is currently wasted on cancer therapies that do not work, giving the example of five new oral treatments for kidney cancer, which cost about $10,000 per month, but often do not substantially change patient outcomes. "If you talk to a doctor who treats renal cell carcinoma, they'll tell you they intend to use every single one of these drugs as long as the patient's alive, but I have treated a lot of renal cell carcinoma and some of these drugs just never worked if you used them as third-, fourth-, or fifth-line options," Kolodziej said.

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Many countries in Europe use value-based pricing. Danzon noted that in these countries, when the price manufacturers want to charge does not meet the payer's threshold value, the manufacturer often pays for a set number of doses per patient with the payer then paying for remaining doses for patients who respond. Alternatively, there is a cap on what the payer would have to pay, and if the patient needs more than a certain number of doses, the manufacturer pays those excessive costs. Some also use reimbursement with evidence development after drugs enter the market. The prices of those drugs are then adjusted after the evidence is assessed. Such risk- and cost-sharing agreements are based on outcomes and are more complex and costly to implement, Danzon pointed out.

With value-based pricing, innovation would be rewarded if it results in a more effective drug, Danzon noted. "A drug that delivers additional health benefits or additional cost savings would get a higher price," she said. She added that the pricing proposal would not cut current U.S. drug prices because it would benchmark new drug prices to existing drug prices. But the initiation of value-based pricing would constrain the growth of prices going forward. She also stressed that adopting such a pricing scheme would not be that difficult given that "we are already well into trying to measure outcomes so it would be relatively simple to move to measuring value in terms of outcome per dollar spent."

But some participants were skeptical about such a pricing system given that there is a lack of relevant data on the value of treatments. Outcomes data collected from the ideal treatment population enrolled in clinical trials often do not reflect outcomes in typical patients who often have comorbidities or other factors that dampen their responses noted Richard Schilsky, chief medical officer, ASCO. "What happens when the incremental benefit in real-world patients is less than the evidence that is demonstrable in clinical trials, which often will be the case?" he asked. Olson noted that the state of Oregon has an evidence-grading process that helps decide what levels of evidence should carry more weight than others and considers other evidence besides those reaped from randomized clinical trials. Also, the more patients resemble those used in clinical trials, the more likely the state will cover the cost of their treatments, he said, with performance status given priority as the key indicator used to justify denying treatment coverage. In his presentation, Olson described the approaches used in Oregon's value-based pricing system for cancer care (see Box 5).

Bastian noted that less than 40 percent of oncology drugs are brought to the market with comparative effectiveness data (Goldberg et al., 2011).

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That, in conjunction with the increasing recognition of the heterogeneity of patient response to cancer drugs based on tumor characteristics, means there is insufficient data on which to make comparative value judgments on cancer drugs, according to Bastian.

Ganz added that there is a lack of patient-reported outcome data and limited clinical trial enrollment of the elderly population most likely to receive cancer drugs. There is also a lack of postmarketing data from manufacturers. This makes it difficult for payers, physicians, and patients to assess the value of various treatments. "Manufacturers don't have any data that are meaningful to us in practice," she said.

Bastian pointed out that a number of new initiatives, such as the Patient-Centered Outcomes Research Institute (PCORI), are beginning to institutionalize measurements of more relevant patient-centered outcomes, and there is increasing infrastructure support for comparative effectiveness research. He noted that Genentech announced a major partnership with the global online patient network PatientsLikeMe to develop innovative ways of studying patients' real-world experience with disease and treatment (Genentech, 2014).

Feeley agreed that there is a need to measure and report outcomes and make them publicly available. "The whole public reporting system in cancer care now is absolutely abysmal and mainly reports processes of care. Even the National Quality Forum has no meaningful measures for outcomes of cancer care and we have to transform that. This will be 5 to 10 years of work, but we have to be honest about being able to publicly report our outcomes and compete on price," he said.

Schilsky noted that cancer drugs are typically introduced into the marketplace after a study in patients with advanced disease in whom the value is likely to be the most difficult to demonstrate and the comparator may often be best supportive care or placebo. Thus, it is difficult to know what the incremental benefit is compared to the prevailing therapies that are otherwise available. Over time these drugs are tested in earlier-stage cancer populations and their true benefit may be revealed in those studies, but not until many years after they first come on the market. Bastian concurred that "cancer is special in the sense that drugs are coming on the market with early, immature datasets, with some launched solely with Phase 2 data alone." Schilsky asked, "So how can one continually adjust price as new evidence becomes available in new patient populations with a value-based pricing scheme?"

Danzon responded that there may be potential for using some sort

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BOX 5
Oregon Health Plan

Kevin Olson reported on the Oregon Health Plan put forward by the Oregon Health Evidence Review Commission, an organization formed to guide the state's Medicaid program. After receiving input from all stakeholders, the state of Oregon decided to meet Medicaid budget constraints by reducing benefits rather than limiting the number of people eligible for Medicaid, or by reducing payments below the cost of delivering care. This required prioritizing coverage using the best evidence of clinical effectiveness, which became the charge of the Oregon Health Evidence Review Commission. "The whole goal was to expand coverage to a larger population of the working poor by funding only the most effective therapies. The key take-home was that there was no guaranteed coverage for all standard therapies," Olson stressed. As far as cancer was concerned, Oregon Medicaid covered treatments given with the intent to cure cancers, although the bar for such treatments was low, as they just had to improve by 5 percent the 5-year survival rate. However, implementation was inconsistent as there were no codes for curable versus incurable cancer.

When it became apparent that cancer costs were escalating at an unsustainable rate, and that cancer spending was occurring disproportionately in the past 6 months of life where it would not be of greatest value, Oregon Medicaid asked the Health Evidence Review Commission (then called the Oregon Health Services Commission) to clarify the most appropriate treatment of cancer and provide a "roadmap for a medical plan director working for Medicaid to deny [coverage of low value] care," Olson said.

This commission recommended that palliative care always be covered, but emphasized the fact that patients with limited performance status[a] are not likely to benefit from chemotherapy. Consequently it created a tiered approach to coverage based on life expectancy, which expanded the list of benefits because prior to the guidance, no one with incurable cancer had coverage for any chemotherapy treatments. Recognizing that money spent unwisely would result in others with curable cancer, pregnant women, and children not receiving the care they need because of the limited Medicaid funds, the Commission developed strict guidelines that limited who qualified for chemotherapy, such as not covering treatment with intent to prolong survival for patients with an expected median survival of less than 6 months with or without treatment, but covering such treatment for all patients with an expected median survival of greater than 2 years. "The goal was to exclude coverage for marginally effective drugs that might add 2, 3, or 4 weeks of life to somebody who only had 6 months," said Olson.

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Several challenges to the guidance surfaced after it was released and that led to modifications. These challenges included difficulties in assessing how much treatments improve overall survival. Olson noted that there was a lack of randomized controlled clinical trials to assess every clinical situation, as well as increasing use of progression-free survival by the Food and Drug Administration in approving drugs for use in patients with terminal illness. In addition, crossover effects in many trials obscure the published outcomes on overall survival.

Then the Patient Protection and Affordable Care Act (ACA) was passed with the provision that treatment decisions cannot be based on life expectancy. The ACA expanded the budget of Medicaid to allow coverage of more patients, but with the expectation that savings from more effective care delivery will offset less federal money in the future, so the expansion could be sustainably affordable. As a result, the Oregon Health Evidence Review Commission convened another task force last year, which modified the previous guideline so that now treatment with intent to prolong survival is not covered for patients with:

- Progressive metastatic cancer and severe comorbidities unrelated to the cancer that result in significant impairment in two or more major organ systems which would affect efficacy and/or toxicity of therapy, or
- A continued decline, in spite of best available therapy, with a nonreversible performance score of <50 percent (as defined by Karnofsky and Burchenal, 1949) or with performance score of 3 or higher (as defined by the Eastern Cooperative Oncology Group; Oken et al., 1982), which are not due to a preexisting disability.

"In other words if you never would have qualified for the studies that the oncologists were using to justify the treatment in the first place, then Oregon Medicaid would not cover it either," Olson said. "Patients who anybody could see are not doing well with therapy would be ineligible for coverage."

But treatment with intent to relieve symptoms or to improve quality of life is still a covered treatment, and there must be evidence that there was a discussion with the patient regarding treatment goals, prognosis, side effects, and reasonable expectations of efficacy. The end result relied heavily on NCCN [National Comprehensive Cancer Network] guidelines, but Olson added that "most of us would say that they are not specific enough to really give them a lot of teeth."

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[a] Performance status is an attempt to quantify cancer patients' general well-being and activities of daily life.

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of modeling that could enable planning for the price to adjust within certain parameters over time and revisiting the pricing at defined periods. "One good thing we have going for us is that with 'big data mining,' the evidence is going to become more readily available. So some combination of postlaunch adjustment and using modeling initially at launch to get an approximately right price would be a reasonable way to go."

There was also some discussion about the need to balance incentives and costs to facilitate innovation. Bastian cautioned against creating price controls or policies that might impede the innovations that could ultimately reduce cost or dramatically change patient outcomes for the long term. Chalkidou added "Innovation is important, but it has to be innovation that we can afford. If health systems can no longer afford innovation, then patients here right now will suffer because they won't be getting access to the treatment they need, as well as patients in the future because health systems will cease to fund innovation the way we've known it. So something has to give. We can't just continue innovating at a cost that's unaffordable."

WRAP-UP

Throughout the workshop, many participants emphasized the unsustainability of the high costs of cancer care, and how that affects not only individual patients, but also society at large. In his summary of the proceedings, Bach noted "We are paying a very high premium in the United States to avoid some conversations that other countries were willing to have about the goals of health care and whether they will fit in the budget."

Several workshop participants suggested ways to contain cancer care costs, including innovative ways to deliver, assess, and pay for cancer care, and helping patients understand the costs of their treatments. Some of these proposals are currently being developed or tested in pilot programs. Bach pointed out that though these solutions may be attractive, they will require a culture change to institute them in this country. But such change may occur out of necessity if health care costs continue to climb.

Hammelman also stressed that "When we look at these new payment models coming out, we have to remind ourselves that whatever we build in the future will be based on what we do today. If there are discrepancies or disparities in today's market, we're only going to continue them, unless we really understand what those disparities are and we address them."

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Appendix

Workshop Statement of Task and Agenda

STATEMENT OF TASK

An ad hoc committee will plan and host a 1.5-day public workshop that will feature panel discussions and invited presentations. Workshop participants will examine patient access to cancer therapies, with emphasis on cancer drug pricing, inflation in the cost of cancer drugs, differences among practice settings, and insurance benefit design. Participants will be invited to discuss topics that may include

- Structural factors influencing drug pricing, such as patents (regulated monopolies), the role of health insurance, and information asymmetries between patients and providers;
- Policy factors, such as cancer drug reimbursement and cost-sharing policies (e.g., co-pays and coinsurance), Medicare reimbursement policies and the legislative limitations on those policies, and state laws prohibiting restrictions on oncology drug prescribing;
- Cancer drug distribution channels and access programs, such as the 340B program and co-pay assistance programs; and
- Changing economics of oncology practice and the historic reliance on office-based drug prescribing for revenues.

The committee will develop the agenda for the workshop sessions, select and invite speakers and discussants, and moderate the discussions. An

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individually authored workshop summary will be prepared by a designated rapporteur based on the information gathered and the discussions held during the workshop in accordance with institutional policy and procedures.

AGENDA

June 9, 2014

7:30 am **Registration**

8:00 am **Welcome from the Institute of Medicine's National Cancer Policy Forum**
Michael Caligiuri, The Ohio State University Comprehensive Cancer Center
Chair, National Cancer Policy Forum

Overview of the Workshop
Peter B. Bach, Memorial Sloan Kettering Cancer Center
Planning Committee Chair

8:15 am **Session 1: Trends in Oncology Care**
Moderator: Barry Fortner, ION Solutions

Escalating drug prices
- Kalipso Chalkidou, NICE International (via phone)

Consolidation of practices into hospital partnerships/ changing landscape of oncology practice
- Bruce Gould, Community Oncology Alliance

The financial toxicity of cancer treatment: Can patients cope with costs?
- Yousuf Zafar, Duke University Medical School, Duke Cancer Center

Drug shortages
- Peyton Howell, AmerisourceBergen

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CMS reimbursement policies in oncology
- Marc Hartstein, Centers for Medicare & Medicaid Services (CMS)

Panel Discussion

10:45 am Break

11:00 am Keynote Address: Pharma pricing practices and the case for market self-correction
- Alex Bastian, GfK Custom Research, LLC

11:30 am Lunch Break

12:15 pm Session 2: Paradoxes of Cancer Drug Reimbursement
Moderator: Jeffery Ward, Swedish Cancer Institute

Brand name drugs vs. generics
- Jeffrey Peppercorn, Duke University Medical School

Oral vs. IV drugs
- Lee Newcomer, UnitedHealth Group

340B vs. everyone else
- Rena Conti, University of Chicago

Private practice vs. hospital-based care
- Eric Hammelman, Avalere Health

Variable co-insurance and premiums
- Michael Kolodziej, Aetna

Panel Discussion

2:45 pm Break

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3:00 pm **Session 3: Value and Policy Options**
Moderator: Scott Ramsey, Fred Hutchinson Cancer Center

The patient perspective
- Shelley Fuld Nasso, National Coalition for Cancer Survivorship

Value-based insurance design
- Mark Fendrick, University of Michigan Center for Value Based Insurance Design

Bundling of payments
- Thomas Feeley, The University of Texas MD Anderson Cancer Center

Value-based drug pricing
- Patricia Danzon, Wharton School, University of Pennsylvania

Lessons from the Health Evidence Review Commission, Oregon Health Authority
- Kevin Olson, Providence Cancer Center

Panel Discussion

5:30 pm **Adjourn**

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