

Turning The Tide AGAINST CANCER

Through Sustained Medical Innovation

A Pathway for Change: Supporting the Shift to Patient-Centered Cancer Research and Care and Addressing Value and Cost of Cancer Care

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Introduction

Incredible strides have been made in biomedical research and oncology care and as a result, the percentage of the U.S. population living with, through, or beyond cancer has more than tripled since the U.S. Congress passed the National Cancer Act in 1971.¹ For many patients, what was once an acute, life-threatening diagnosis has been transformed into a manageable, chronic condition. Our expanding knowledge of the human genome and the biology of cancer has supported the development of highly targeted therapies tailored to the unique features of a patient's disease, helping patients to live longer and better lives. Moreover, today's healthcare system is testing new approaches to care delivery that have the potential to produce higher quality, more patient-centered medical care.

However, significant gaps remain and many cancer patients still face significant unmet needs. More than 1.6 million people in the United States are projected to receive a cancer diagnosis in 2014, and more than 585,000 are expected to die from the disease.² Increases in the demand for care, along with inconsistent quality of care and unsustainable costs, have led to growing pressure for cost containment. With the aging of our population, the number of new cancer cases in the United States is expected to rise significantly, reaching almost 2.4 million by 2035.³ Unless more effective strategies for preventing, detecting, and treating cancer can be developed, it will not be long before cancer overtakes heart disease as the leading cause of death in the United States. This growing population of cancer patients will place strains on access and the cost for preventative, screening, and treatment services. And with efforts across the healthcare environment to contain costs, there is concern that innovative interventions may not be adequately reimbursed in the future.

Progress in oncology is at a critical juncture. Many in the cancer community believe that continued scientific progress will play a central role in meeting the challenge of rising cancer care costs as well as the challenge of patients' unmet needs.⁴ If we are to capitalize on research advancements to ensure that patients benefit from life-changing developments, we must ensure that health policy incentivizes the development of innovative interventions and the delivery of high-quality, patient-centered cancer care. Policies are needed that align scientific progress with the evolving way research is conducted and care is delivered.

Background

In 2011, the Personalized Medicine Coalition,^I the American Association for Cancer Research,^{II} and Feinstein Kean Healthcare^{III} launched the Turning the Tide Against Cancer initiative, a national effort that aims to identify options that will sustain medical innovation, while addressing the issue of rising healthcare costs. In June 2012, the initiative held its first national conference, “Turning the Tide Against Cancer Through Sustained Medical Innovation.” As part of this conference, six goals for sustaining innovation in an era of cost containment were identified. They included: support the shift to patient-centered care in oncology; align comparative effectiveness research (CER) and health technology assessment (HTA) with the patient and the science; support the development of molecular diagnostics; encourage partnerships and collaborations; support a continuous learning healthcare system; and engage society in cost of cancer conversations. These goals were further discussed at the initiative’s October 2013 Roundtable and in a March 2014 *Clinical Cancer Research* article by many of the Turning the Tide Against Cancer leaders.^V

This year, the Turning the Tide Against Cancer initiative commissioned Avalere Health to facilitate an expert working group in order to identify actionable policy options that will support the delivery of patient-centered, high-value oncology research and care and guide future Turning the Tide Against Cancer activities.^{IV} The expert working group included participants from a range of disciplines, including academic medicine, health plans, pharmaceutical manufacturers, health policy experts, practicing physicians, and patient representatives.^V

The working group focused on two key themes that have emerged from the initiative’s work over the past two years and are directly relevant to the challenges of sustaining continued innovation and rising pressure for cost containment: (1) How to foster a shift to patient-centeredness in cancer research and care delivery and (2) How to address cost and value in oncology in ways that align with patient-centeredness and scientific progress.^{6,7}

Methodology

To identify the policy options presented and generally supported by the collective working group, Avalere reviewed select white and grey literature available in the public domain that focused on oncology policy in order to identify early efforts that move toward patient-centered cancer research and care and address value and cost. Avalere scanned those articles and developed a comprehensive list of potential oncology-related policy options being considered today by the broader cancer and healthcare communities. Sources reviewed included peer-reviewed publications and white papers from organizations such as the American Society of Clinical Oncology, Commonwealth Fund, Institute of Medicine, Pew Research Center, Kaiser Permanente Institute for Health Policy, and Medicare Payment Advisory Commission (MedPAC). Avalere also interviewed key opinion leaders for input and recommendations. As part of this exercise, Avalere included only policy options that fell into the two areas of patient-centered research and care and cost and value, as noted above.

^I Additional information can be found at: www.personalizedmedicinecoalition.org

^{II} Additional information can be found at: www.aacr.org

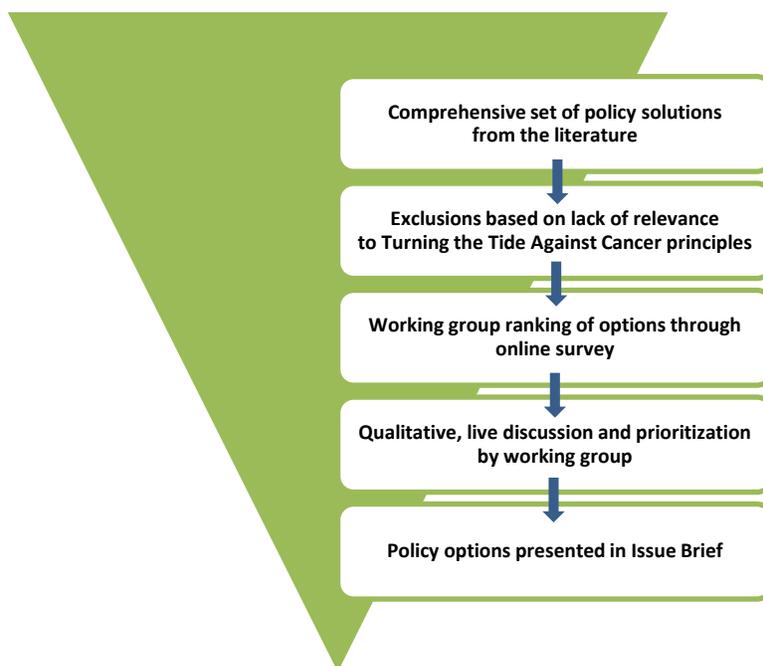
^{III} Additional information can be found at: www.fkhealth.com

^{IV} The views reflected in this paper are not necessarily the views of individual members of the working group or the organizations that they represent.

^V See Appendix A for a full list of working group participants.

The initial set of options identified by Avalere provided a baseline for working group participants to assess the benefits and risks of each option and to add additional options as part of a prioritization process. Through an online survey, virtual group discussion, and key informant interviews, working group members ranked and provided additional context and feedback. The quantitative and qualitative output was presented to and discussed by the working group to assess the validity and relevance of each option, and placement within the working group policy principles (see **Figure 1**). The results are presented in this Issue Brief.

Figure 1: Identifying Actionable Policy Options



Policy Principles to Turn the Tide Against Cancer

1. Supporting the Shift to Patient-Centered Research and Cancer Care

Principle 1A: Patients should be engaged at relevant points throughout the research and care delivery processes.

- Research sponsors and research organizations should engage patient advocacy organizations and patients in defining endpoints and outcomes most relevant to them and their caregivers.
- Payment for and the delivery of oncology care should support patient-centeredness and patient engagement.
- Information collected from patients should be used to inform patient-provider interactions.

Principle 1B: The national health information infrastructure should capture information relevant and meaningful to clinicians, patients, and their caregivers.

- Emerging platforms for data generation and collection through electronic health records (EHR) and other health information technologies (HIT) should be interoperable and capture meaningful, patient-centered results, including patient-reported outcomes.
- Decision-making (e.g., shared decision-making, clinical decision support) tools should reflect clinical best practice and support individualized treatment decisions, including patient preferences, and provide a feedback loop to support the patient-clinician interaction.
- Information collected in the context of a healthcare interaction should, by consent, be available to be used for research purposes.

2. Addressing Value and Cost of Cancer Care

Principle 2A: Assessing, communicating, and incentivizing value in payment and delivery innovations should encourage efficiencies and support care coordination and patient-centered care consistent with clinical best practices.

- Oncology quality measures that take into account both clinical and patient-reported outcomes should be developed and utilized. Measures should evolve and keep pace with research and standards of care.
- Reporting of patient perspectives should adequately reflect differences in perspectives on cost and value and variability in value among patients.
- Proposed payment reforms should support the shift to patient-centeredness in cancer care through services such as oncology patient-centered medical homes, patient navigation, and care coordination.
- Physician and patient communication should take into account patients' clinical and economic circumstances, and be aligned with best practice.

Principle 2B: Methods and tools for assessing the value of healthcare interventions should reflect heterogeneity in patient populations and be created transparently and with engagement from relevant stakeholders.

- Tools for assessing the value of medical interventions (e.g., oncology comparative effectiveness research and health technology assessment and health service research) should be designed to capture the outcomes that matter to patients in real time (e.g., patient registries, new HIT formats).
- Methods for assessing the value of medical interventions should be transparent, apply the best evidence available, and take into account the needs and individual circumstances of patients.

Based on these guiding principles, the working group identified a series of specific policy and regulatory options described below. The working group sought to identify tangible next steps to advance each of these options and to place them in the broader framework, described above, of accelerating progress and fostering patient-centeredness in an era of growing cost containment.

The working group recognizes that this list is far from exhaustive. Due to time and capacity limitations, the working group could not address all potential issues that were identified. For example, prior work by the Turning the Tide Against Cancer initiative and others has identified digital health and continuous learning as important opportunities that are not directly addressed in this paper, though the working group sought to recognize places where they could be leveraged in the cancer care ecosystem. This may be a fruitful area for future work.

In addition, several more controversial proposals are not included because full consensus among the working group could not be reached. Finally, as the working group began its work, it became apparent that many members were prioritizing regulatory reforms at the U.S. Food and Drug Administration (FDA) as well as reforms in healthcare policy and care delivery. While not a primary focus of this paper, the working group did seek to include several leading-edge regulatory options on which there was broad agreement. This may be an additional area where progress can be made in future work.

The policy options outlined below reflect the work of a broad range of leaders representing diverse stakeholders in oncology. They are intended to represent a set of initial, meaningful steps that can be taken in support of cancer research and care that is innovative, efficient, and patient-centered.

Policy Options to Support Patient-Centered Cancer Research and Care and to Address Cost and Value in Cancer Care

1. Congress should fund and the National Institutes of Health (NIH) should implement public/private partnerships to encourage the use and acceptance of innovative clinical trial designs that promote efficiency in drug development by, for example, enabling simultaneous study of multiple drug candidates.
2. FDA should promote the modernization of the framework for bringing new medicines to patients by facilitating and encouraging the use of innovative approaches to drug development and regulatory review, including the use of novel clinical trial designs, integration and consideration of patient perspective information in regulatory benefit-risk assessments, and use of observational research for pre- and post-market regulatory decision-making.
3. FDA should continue making progress in defining and applying a clear, efficient, and coordinated review process for personalized medicine products.
4. The U.S. Department of Health and Human Services (HHS) should establish a cross-department work group to identify opportunities to enhance data transparency and sharing in support of innovation in oncology, including for data related to pre-competitive collaborations, clinical trial data, and federal and state electronic data sets (e.g., Medicare claims data). Policies should maximize transparency and sharing of high-quality data, while supporting strong standards for protecting patient confidentiality and commercial confidential data.
5. Congress should provide funding to support the development and updating of quality and performance measures for cancer care by private sector organizations (including oncology and related medical specialty societies and organizations with expertise in patient experience and patient-reported outcomes measures) through transparent procedures that include multi-stakeholder endorsement. The Center for Medicare and Medicaid Innovation (CMMI) should require use of robust, clinically-driven, and endorsed clinical quality and patient-focused measures in alternative oncology payment models.
6. HHS and states should ensure patient access to quality and affordable care in federal and state health exchanges by requiring broader coverage of cancer services and drugs and assuring adequate networks of cancer providers.

7. CMMI should prioritize additional funding for Oncology Patient-Centered Medical Home (OPCMH) demonstrations, with a focus on supporting patient navigation, access to care providers and treatment options, and personalized, evidence-based treatment plans, using tools such as shared decision-making. OPCMHs should incentivize adoption of advanced electronic health records and informatics and be evaluated against clear, patient-centered metrics, including measures of care quality and patient experience, and access to medically appropriate treatments and care providers.
8. Medicare reimbursement policies should support innovative practice models to improve patient access and support patient engagement. These policies may include payment for telemedicine, oncology nursing support, visiting consultants, e-mail, and use of mobile devices.
9. The Centers for Medicare and Medicaid Services (CMS) should adopt more specific codes (developed by the American Medical Association [AMA]) to appropriately capture the complexity of cancer tests and services and ensure appropriate reimbursement, including for molecular and personalized medicine testing as well as palliative care.
10. CMS should ensure that cancer clinical pathways or similar decision support tools used to guide clinical decision-making are transparent to beneficiaries and the public; developed through a physician-driven process that includes patient input; and meet minimum standards for clinical appropriateness, timeliness, and patient-centeredness. The Institute of Medicine (IOM) should consider convening a multi-stakeholder committee to make recommendations on standards for clinical pathways, including transparency, evidence quality, and incorporation of genetics tests and personalized medicine.
11. Federal health agencies, including HHS and the Office of the National Coordinator for Health Information Technology (ONC), should support oncology decision support tools that are timely, clinically appropriate, and patient-centered. In particular, ONC should propose certification standards for electronic health records (EHRs) to improve the frequency of incorporating compendia updates and to ensure that clinical decision support tools meet baseline standards for transparency, strength of evidence, and timeliness to ensure they reflect optimal cancer care, incorporate individualized patient preferences and needs, and keep pace with changes in research and treatment.
12. Building on existing efforts, IOM should convene a multi-stakeholder committee and develop a report on how to define and measure value in oncology care that addresses dynamics previously identified by Turning the Tide Against Cancer leaders⁸ – variability in definitions of value within and among stakeholders and over time – so that methods for assessing value align with the needs of patients and continued scientific progress.
13. The Patient-Centered Outcomes Research Institute (PCORI) should continue to support research to evaluate and identify innovative, effective methods for the use of decision support tools to best communicate to patients and caregivers benefit, risk, and uncertainty in evidence. Research should include consideration of patient preference in treatment decision-making.

Option 1: Congress should fund and NIH should implement public/private partnerships to encourage the use and acceptance of innovative clinical trial designs that promote efficiency in drug development by, for example, enabling simultaneous study of multiple drug candidates.

Developing and refining innovative approaches to conducting cancer research is essential if we are to continue to make progress against cancer. For example, innovative clinical trial designs (e.g., I-SPY2, Lung Cancer Master Protocol)^{9,10} show potential for rapid-cycle, adaptive evidence generation and implementation by using early study results to inform treatment assignments for subsequent patients. Public/private research partnerships and innovative trial designs can play an important role in accelerating the discovery of life-saving and life-improving cancer therapies. The NIH and National Cancer Institute (NCI) can play a leading role as conveners and facilitators of new, innovative collaborations to advance adaptive clinical trials and other novel approaches to the development of cancer therapeutics and in generating insights on clinically meaningful outcomes for patients. The Institutes should continue to support such work – without cannibalizing existing federal research funding or other important efforts.

Option 2: FDA should promote the modernization of the framework for bringing new medicines to patients by facilitating and encouraging the use of innovative approaches to drug development and regulatory review, including the use of novel clinical trial designs, integration and consideration of patient perspective information in regulatory benefit-risk assessments, and use of observational research for pre- and post-market regulatory decision-making.

Working group participants agree that large, randomized clinical trial (RCT)-based drug development may not be the only appropriate model for 21st century research, particularly for developing new medical products for serious or life-threatening diseases and unmet medical needs. Reforms to the research and regulatory infrastructure and process must encourage and facilitate the development of new therapies to keep pace with scientific progress. There are a number of opportunities to leverage existing advances – both in scientific knowledge and in research methodologies/trial designs – to encourage innovation. Numerous organizations are beginning to partner to capitalize on these advances and leverage shared knowledge, including: Pfizer and 23andMe;¹¹ Genentech and PatientsLikeMe;¹² and Boehringer Ingelheim and the Duke Clinical Research Institute.¹³

Larger and more diverse data sets are being generated through the real world use of clinical and care delivery interventions and are subsequently being accessed to support healthcare decision-making. FDA should consider how to better utilize analyses based on real-world electronic data sets in the full scope of its decision-making, including for considering applications for supplemental indications, revising a drug's labeling to reflect patient outcomes, improving the scientific basis for defining the nature of or need for Risk Evaluation and Mitigation Strategies,¹⁴ or fulfilling post-marketing commitments.

In addition, patient-centered cancer care depends on understanding how patients are engaged in their own treatment decisions, in order to identify areas of unmet need and study designs that best capture the endpoints and outcomes that are meaningful to patients. Through its Patient-Focused Drug Development (PFDD) initiative,¹⁵ FDA has embarked on an effort to develop a broad, systematic approach to gathering patients' perspectives on the severity of their diseases and currently available treatment options. Though it is not yet clear how the Agency will do so, the most recent reauthorization of the Prescription Drug User Fee Act (PDUFA V) commits the FDA to using this information to inform its review of medical product applications. The working group encourages

FDA and other stakeholders to continue to actively consider the patient perspective in all aspects of the research and approval process.

Option 3: FDA should continue making progress in defining and applying a clear, efficient, and coordinated review process for personalized medicine products.

The FDA's recently finalized guidance on *In Vitro* Companion Diagnostic Devices is an example of the steps the FDA is taking to improve the regulatory framework for personalized medicine products. This guidance clarifies the process through which FDA will approve or clear a companion diagnostic in coordination with its corresponding therapeutic.¹⁶ Further, the final guidance states that the FDA can approve a therapeutic product even if an *in vitro* companion diagnostic device is not yet approved or cleared if the therapeutic product is intended to treat a serious or life-threatening condition such as cancer.¹⁷

Coordinated review has been carried out very effectively for many oncology products, and several targeted therapies were approved in coordination with their companion diagnostic even before finalization of the guidance. However, some working group participants indicated that this process could still be improved, particularly for non-oncology therapeutic areas. Specifically, working group participants expressed a need for greater coordination and transparency among the drug and diagnostic sponsors and the respective FDA centers, both before an application is submitted and throughout the review process. Enhanced coordination and transparency of the process will help ensure consistent and timely reviews of these products.

Some working group participants also noted that additional guidance would facilitate development of diagnostic tests that utilize next generation sequencing technologies, especially as these tests will become more widely utilized in the oncology clinic. The working group was also encouraged by the Agency indicating that it will continue its open dialogue with all stakeholders as it explores an optimal framework to oversee laboratory developed tests.

Option 4: HHS should establish a cross-department work group to identify opportunities to enhance data transparency and sharing in support of innovation in oncology, including for data related to pre-competitive collaborations, clinical trial data, and federal and state electronic data sets (e.g., Medicare claims data). Policies should maximize transparency and sharing of high-quality data, while supporting strong standards for protecting patient confidentiality and commercial confidential data.

Fostering greater availability of health data, including data from pre-competitive collaborations, clinical trials, and federal- and state-level health data sets (e.g., Medicare claims data and state multi-payer claims databases, respectively), holds potential for accelerating progress and improving delivery of care for cancer patients. For example, sharing data from studies aimed at elucidating new biomarkers could provide insight into potentially duplicative efforts or failed studies that would be useful to other research teams and ultimately accelerate progress in the field.¹⁸

At the same time, stakeholders face significant barriers to advancing data sharing and transparency. It is important to address these barriers while maintaining strong protections for patient confidentiality and commercial confidential information.

One example of an approach to data sharing is the Beacon Community Program, which is exploring how data sharing across multiple health system communities can help achieve their clinical transformation objectives.¹⁹ While data sharing can support community-level care

improvement, opening data access to stakeholders outside of the provider sector or a specific community can also facilitate broader awareness and application of important insights into subsequent research, and accelerate the development of innovative therapies that address the full spectrum of patient needs.

Efforts are presently underway to expand sharing of clinical trial data in a manner that respects patient privacy, the integrity of regulatory decision-making, and incentives to invest in innovation.²⁰ A number of biopharmaceutical and medical device manufacturers, including Eli Lilly and Company,²¹ Johnson & Johnson,²² Medtronic,²³ and GlaxoSmithKline,^{24,25} have already begun to open data access for public review and adjudication of the data by a third party.

Finally, electronic health data sets supported by federal and state agencies are becoming increasingly robust and represent an important opportunity for rapid learning in oncology and healthcare more generally. These data sets (e.g., Medicare claims data, state multi-payer claims databases, and the National Patient-Centered Clinical Research Network being built by the Patient-Centered Outcomes Research Institute) should be governed by clear, consistent data access policies that allow qualified researchers from across the cancer ecosystem to access data sets for appropriate research purposes.

Working group participants believed that current efforts would be well complemented by the establishment of a work group composed of representatives from HHS, industry, academia, and patient groups to consider and make recommendations to HHS and Congress about incentives that encourage appropriate sharing of clinical trial data for qualified research, provider-generated data, and biomarker information.

Option 5: Congress should provide funding to support the development and updating of quality and performance measures for cancer care by private sector organizations (including oncology and related medical specialty societies and organizations with expertise in patient experience and patient-reported outcomes measures) through transparent procedures that include multi-stakeholder endorsement. CMMI should require use of robust, clinically-driven, and endorsed clinical quality and patient-focused measures in alternative oncology payment models.

There is presently a lack of agreement on how to define and measure “quality” in cancer care, but there also is broad recognition that significant gaps exist in the current stock of cancer quality measures. Quality metrics across the healthcare continuum are often inconsistent with the way quality is understood by patients: working group participants noted, for example, that there has been little alignment of outcomes evaluated in clinical trials with outcomes assessed by quality and performance measures. Participants recognized the need for quality and performance measures that reflect and keep pace with clinical best practice and also recognize important aspects of the patient and caregiver experience.

While some work has begun in this space, notably through the National Quality Forum (NQF)-convened Measures Applications Partnership (MAP), which has helped CMS prioritize measure gap areas, more remains to be done.²⁶ In its 2013 report, *Delivering High-Quality Cancer Care: Charting a New Course for a System in Crisis*, the IOM identified significant gaps in the existing quality measure sets and recommended that HHS prioritize development of meaningful cancer outcome measures for use in public reporting.²⁷ Additional government funding is needed to close gaps in measures related to a broader range of clinical outcomes that are meaningful to patients,

as well as outcomes related to patient preference and quality of life, screening and prevention, and patient communication and care coordination. These efforts should build on existing measure development efforts that are already underway by oncology professional societies and other clinical organizations (e.g., ASCO's Quality Oncology Practice Initiative).

In addition, working group participants noted that the development and implementation of measures must involve multi-stakeholder input and reflect the most current advances in clinical treatment. More outcomes-based measures (versus process of care measures) are needed to assess clinical outcomes in addition to pain, functional status, and other domains of patient-reported outcomes (PROs).²⁸ Participants expressed the increasing importance of PROs in informing payer decision-making, and the need for aligning use of PRO-based performance measures (PRO-PMs) with supporting use of PRO endpoints in the pre-market studies that are needed to secure FDA approval of a new medicine. In addition, discussants recognized the complexity and administrative burden of having different payers and programs including different sets of measures and the need for greater harmonization of the measures that are more important to ensuring high-quality, patient-centered care.

Option 6: HHS and states should ensure patient access to quality and affordable care in federal and state health exchanges by requiring broader coverage of cancer services and drugs and assuring adequate networks of cancer providers.

Members of the working group agree that ensuring patient access to quality and affordable cancer care – both at the treatment level and provider level – is essential. There are concerns, however, that the federal and state health exchanges implemented following the passage of the Affordable Care Act (ACA) may be restricting patient access to cancer treatment and services through the nation's leading cancer centers.²⁹ Under the current rules, coverage is dictated by state-defined benchmark plans organized by level of plan generosity. While this approach affords states and plans unprecedented flexibility to design benefits and set premiums, it may pose challenges for patients. In fact, a recent Avalere analysis reveals that exchange plans impose utilization management on self-administered oncology drugs at a higher rate than employer plans (43 percent and 30 percent, respectively),³⁰ potentially limiting patient access to needed treatments. Moreover, seeking to keep premiums low in the health exchanges, most insurers have designed narrow provider networks, some of which exclude leading cancer centers. For example, insurers such as Anthem (formerly WellPoint), UnitedHealthcare, Aetna, and smaller local carriers are increasingly dropping nationally accredited cancer-specific centers from their networks.³¹ Federal and state policymakers should monitor and respond to potential access barriers through respective oversight and regulations.

Option 7: CMMI should prioritize additional funding for Oncology Patient-Centered Medical Home (OPCMH) demonstrations, with a focus on supporting patient navigation, access to care providers and treatment options, and personalized, evidence-based treatment plans, using tools such as shared decision-making. OPCMHs should incentivize adoption of advanced electronic health records and informatics and be evaluated against clear, patient-centered metrics, including measures of care quality and patient experience, and access to medically appropriate treatments and care providers.

The OPCMH model, which emphasizes coordinated, team-based care for a particularly complex and vulnerable patient population,^{32,33} appears well-positioned to meet the Turning the Tide Against Cancer initiative's goal to advance patient-centeredness, innovation, and value in cancer

care. OPCMHs rely on a diverse care team, with strong incentives for shared decision-making and care coordination across disciplines (e.g., pharmacist, primary care provider, oncologist, psychologist) in order for OPCMHs to operate successfully and achieve patient-level cost and quality goals. This approach creates fewer risks for care stinting or for treatment protocol misalignments with personalized medicine and patient-centeredness in oncology than other available models.

CMMI should build on existing OPCMH efforts, such as the Community Oncology Alliance Oncology Medical Home,³⁴ and apply lessons learned when funding additional OPCMHs,³⁵ devoting particular attention to ensuring that they promote optimal delivery of care to individual, highly variable patients. In doing so, it will be important to evaluate OPCMHs' impact on patient care, including patient access to innovative treatments and care approaches. Moreover, it will be critical to ensure that OPCMHs are able to develop personalized care management and treatment pathways and utilize new life-saving products while still employing the most efficient, high-quality treatment options in developing a patient's care management plan.

Option 8: Medicare reimbursement policies should support innovative practice models to improve patient access and support patient engagement. These policies may include payment for telemedicine, oncology nursing support, visiting consultants, e-mail, and use of mobile devices.

New approaches to clinical practice are facilitating more comprehensive care and enabling greater shared decision-making. These models also have the potential to modernize and create new efficiencies in many aspects of care delivery. These approaches include telemedicine, visiting consultants, oncology nursing support, psychosocial support, care planning, email, and use of mobile devices,^{36,37} but there is currently limited reimbursement of such tools and programs, despite a growing evidence base to support their adoption.

While there is potential for these technologies to be adopted within emerging payment and delivery models, there would be benefit to encouraging their adoption outside of these models as well. Policies should utilize innovative information technology and care delivery models to support approaches to shared decision-making that provide timely, accurate information on the range of available tests and treatment options and enable providers and patients to optimize care in support of the individual's treatment goals.

Option 9: CMS should adopt more specific codes (developed by the AMA) to appropriately capture the complexity of cancer tests and services and ensure appropriate reimbursement, including for molecular and personalized medicine testing as well as palliative care.

Clear and concise coding and equitable payment mechanisms are crucial to ensuring that providers are reimbursed for services provided today and are able to invest in tomorrow's innovations. For instance, recent Healthcare Common Procedure Coding System (HCPCS) coding changes have hindered coverage and payment for a number of personalized medicine diagnostic tests. The Protecting Access to Medicare Act of 2014 (PAMA) dictates a new Medicare rate-setting system for clinical diagnostic laboratory tests and mandates a temporary coding structure for new advanced diagnostic laboratory tests (ADLTs) and FDA-approved/cleared tests.³⁸

However, several working group participants noted that the process for obtaining new codes and ensuring coverage and reimbursement for diagnostics related to new personalized medicine treatment options has been extremely cumbersome, has lacked transparency, and has lagged behind need.³⁹ Beyond companion diagnostics, there are a number of other key gaps where improvements in coding are needed. For example, though palliative care is shown to improve the quality of life of patients and family members, as well as the physical and emotional symptoms of cancer and its treatment,⁴⁰ Medicare currently pays for palliative care services only within the hospice benefit – which means that important aspects of palliative care go unaddressed by Medicare.⁴¹ Working group participants also noted the need for improvements in coding in the following areas: use of molecular or companion diagnostics, end-of-life treatment planning, psychosocial distress screening, supportive care, nursing support, test result interpretation and analytics, among others. These improvements should be rapidly advanced by professional groups such as the AMA (currently responsible for the creation of CPT codes) and CMS (currently responsible for the creation of level II HCPCS codes).

Option 10: CMS should ensure that cancer clinical pathways or similar decision support tools used to guide clinical decision-making are transparent to beneficiaries and the public; developed through a physician-driven process that includes patient input; and meet minimum standards for clinical appropriateness, timeliness, and patient-centeredness. The IOM should consider convening a multi-stakeholder committee to make recommendations on standards for clinical pathways, including transparency, evidence quality, and incorporation of genetic tests and personalized medicine.

Stakeholders across the spectrum are testing a range of new approaches to delivering high-quality, affordable healthcare. Many of these approaches are grounded in evidence-based medicine and use the best available information to inform a continuous learning healthcare system.⁴² These include clinical decision support tools, clinical treatment pathways, and medical compendia, which often offer a structure in which providers (particularly those bearing financial risk) can adhere to current standards of care, reduce inappropriate variability in clinical practice, and increase efficiency.⁴³ But without common standards for their development and little harmonization in care approaches across different pathways from different developers,⁴⁴ the working group noted that clinical pathways often cause confusion among physicians; similar challenges likely exist in the implementation of clinical decision support. One participant noted that many different approaches are in development – with “variable methods and variable purposes.” For instance, clinical pathways can be quite sophisticated – based on the best available data and individual patient information – or relatively simple guidelines that can limit treatment options solely because of cost or based on a “typical” patient.⁴⁵ CMS should ensure that clinical pathways used in value-based payment programs meet basic standards for transparency, evidence base, and clinical appropriateness. The development of an evidence-based, consistent, and transparent⁴⁶ approach to developing decision-support tools is critical to achieving widespread acceptance, and could be undertaken by an entity such as IOM, or by a voluntary collaboration among leading health plans, physicians groups, and drug and medical device manufacturers.

Option 11: Federal health agencies, including HHS and ONC, should support oncology decision support tools that are timely, clinically appropriate, and patient-centered. In particular, ONC should propose certification standards for EHRs to improve the frequency of incorporating compendia updates and to ensure that clinical decision support tools meet baseline standards for transparency, strength of evidence, and timeliness to ensure they reflect optimal cancer care, incorporate individualized patient preferences and needs, and keep pace with changes in research and treatment.

There is currently no required timeline or process for EHR vendors to disseminate updates to drug compendia to their customers. As a result, information in EHRs can be out-of-date and prescribers may lack access to the latest product information, including indications, allergies, interactions, and warnings. As today's data infrastructure evolves, it will be important to consider opportunities to incorporate these evidence-based decision tools into EHRs to ensure their feasibility in medical practice.⁴⁷ The working group considered that there may be a role for ONC, which is currently responsible for the development of meaningful use criteria,⁴⁸ to develop certification standards that support the usability, transparency, evidence strength, and timeliness of decision-support tools and any information housed within EHRs (i.e., compendia). ONC should also ensure that EHRs are designed to enable the collection of data that can be used to advance research and patient care (e.g., the collection and analysis of genomic information and patient-reported outcomes can aid in clinical decision-making, as well as accelerate outcomes research more broadly). These decision-support tools can be leveraged to engage patients across the continuum of research and care delivery.

Option 12: Building on existing efforts, IOM should convene a multi-stakeholder committee and develop a report on how to define and measure value in oncology care that addresses dynamics previously identified by Turning the Tide Against Cancer leaders – variability in definitions of value within and among stakeholders and over time – so that methods for assessing value align with the needs of patients and continued scientific progress.

Even as the cost of cancer treatment comes under increased scrutiny, there is growing recognition of the challenges in evaluating and communicating value in ways that are patient-centered, reflect the various dimensions of quality, accommodate differences within and among stakeholder groups (e.g., patient subgroups) in how value is perceived, and take into account continuing advances in research and clinical practice.⁴⁹ Even within a narrow cost framework, there is a trade-off between short-term and long-term costs, and costs that a patient experiences (e.g., cost sharing) versus those imposed on the health system. Patients are often willing to accept such trade-offs (e.g., between incremental survival benefits and toxicity of a therapy), and this willingness may evolve throughout the course of treatment, particularly as patients experience changes in functional status and quality of life. Presently, there is no mechanism or model for understanding and determining (1) what these trade-offs are, (2) how they evolve, and (3) how variances in patient preferences may affect interpretations of value (at an individual level – not just a population health level).

With competing definitions and measurements of value that vary by stakeholder group, working group participants noted the need for a common framework for future discussions and decision-making among payers, physicians, patients, and others involved in treatment decisions. This framework must accommodate patient preference and quality of life and critical factors weighed by patients in making value judgments. Working group participants also noted that understanding of value is continuously evolving as research, science, and clinical practice continually advance. Prior research has noted the extent to which existing evidence-based, decision-support tools are challenged in keeping pace with the rapid rate of change in cancer care;⁵⁰ as a result, the working group advised that these tools should be grounded in the latest clinical evidence, not just what is deemed the current standard of care. This approach will allow for more targeted treatments to be developed.

The IOM's November 2009 workshop summary, *Assessing and Improving Value in Cancer Care*,⁵¹ provides a starting point for renewed efforts to address this issue in light of the significant and ongoing scientific advances that have occurred since then, as well as ongoing policy development. In addition, the ASCO Value in Cancer Care initiative seeks to provide a framework for assessing value that accommodates differences in patient perspectives of value, reflects the value of oncology innovation, and gives oncologists a tool for discussing value with their patients.⁵² Such a framework will need to define the different components that inform a more patient-centered notion of value – including survival, toxicity, harm, symptoms, palliation, convenience, functional status, and other relevant inputs – and weigh clinical value and cost in ways that reflect patient preferences, individual willingness to accept trade-offs, and other needs more broadly.

Option 13: PCORI should continue to support research to evaluate and identify innovative, effective methods for the use of decision support tools to best communicate to patients and caregivers benefit, risk, and uncertainty in evidence. Research should include consideration of patient preference in treatment decision-making.

To advance a patient's understanding of value, working group participants noted that patients will need to access the necessary information to be able to understand and evaluate their various treatment options at their individual (and varying) levels of health literacy. PCORI, which has included Communication and Dissemination as part of its National Priorities and Research Agenda,⁵³ is optimally positioned to identify the appropriate mechanisms to communicate to patients the relative benefits and risks, as well as remaining uncertainties or gaps in the available evidence, associated with potential treatment approaches.^{54,55} PCORI has already funded a significant amount of research to advance shared decision-making approaches and provider/patient decision support tools and should continue to do so.⁵⁶

Conclusion

We are at a crossroads in oncology research and care. Advances in scientific knowledge create great opportunity for continuing improvements in clinical care but increasing pressures to contain costs present potential challenges to further innovation. Federal and state governments play important roles in addressing these challenges, as they fund research, inform standards, and regulate and pay for healthcare. However, working group participants noted that government is often constrained by a range of factors and may not be the best or most appropriate entity to support needed changes. As a result, policy efforts will need to be complemented by the private sector, including through private/independent collaborations. Such efforts may be more nimble in supporting rapid evidence development and translation of this evidence into innovative research and care models that are otherwise challenging for governmental actors to match.

This Issue Brief presents policy options that both public and private stakeholders can adopt to support the shift toward patient-centered cancer research and care while driving to realize value in cancer research and care. We hope the ideas described here provide guidance and catalyze action for individual stakeholder organizations and policymakers to ensure that future investments in research and care delivery will address the evolving needs within the cancer community.

Appendix A

Participants in the working group are listed below. The views reflected in this paper are not necessarily the views of individual members of the working group or of the organizations they represent.

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Craig Burton, Avalere Health (moderator)

Tanisha Carino, Avalere Health (moderator)

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References

- ¹ American Association for Cancer Research. AACR Cancer Progress Report 2014. *Clin Cancer Res* 2014;20(Supplement 1):SI-S112
http://cancerprogressreport.org/2014/Documents/AACR_CPR_2014.pdf
- ² American Association for Cancer Research. AACR Cancer Progress Report 2014. *Clin Cancer Res* 2014;20(Supplement 1):SI-S112
- ³ American Association for Cancer Research. AACR Cancer Progress Report 2014. *Clin Cancer Res* 2014;20(Supplement 1):SI-S112
- ⁴ Abernethy A, Abrahams E, Barker A, et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clin Cancer Res* March 2014. <http://clincancerres.aacrjournals.org/content/20/5/1081.full?keytype=ref&siteid=aaacrjnl&ikey=6zXz3V7qFG.d6>
- ⁵ Abernethy A, Abrahams E, Barker A et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clin Cancer Res* March 2014.
- ⁶ Turning the Tide Against Cancer. Sustaining Progress Against Cancer in an Era of Cost Containment. Discussion Paper. June 2012.
<http://turningthetideagainstcancer.org/sustaining-progress-discussion-paper.pdf>
- ⁷ Abernethy A, Abrahams E, Barker A, et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clin Cancer Res* March 2014.
- ⁸ Abernethy A, Abrahams E, Barker A, et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clin Cancer Res* March 2014.
- ⁹ Turning the Tide Against Cancer. Sustaining Progress Against Cancer in an Era of Cost Containment. Discussion Paper. June 2012.
- ¹⁰ Lung Cancer Master Protocol. Accessed: <http://www.lung-map.org/>
- ¹¹ Avalere Health. 23andMe Partners with Pfizer and Receives NIH Grant. *EBM Insights*. 25 August 2014.
http://ebm.avalerehealth.net/newsletters/205?article_id=948
- ¹² Avalere Health. PatientsLikeMe and Genentech Enter Patient-Centric Research Collaboration. *EBM Insights*. 14 April 2014.
http://ebm.avalerehealth.net/newsletters/187?article_id=871
- ¹³ Avalere Health. Boehringer Ingelheim and Duke Clinical Research Institute Collaborate to Study IPF. *EBM Insights*. 10 February 2014.
http://ebm.avalerehealth.net/newsletters/175?article_id=827
- ¹⁴ FDA Briefing Document. Readjudication of the Rosiglitazone Evaluated for Cardiovascular Outcomes and Regulation of Glycemia in Diabetes Trial (RECORD). Joint Meeting of the Endocrinologic and Metabolic Drugs Advisory Committee and the Drug Safety and Risk Management Advisory Committee.
<http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/EndocrinologicandMetabolicDrugsAdvisoryCommittee/UCM354859.pdf>
- ¹⁵ FDA. Patient-Focused Drug Development: Disease Area Meetings Planned for FY 2013-2015.
<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm>
- ¹⁶ FDA. *In Vitro* Companion Diagnostic Devices: Guidance for Industry and Food and Drug Administration Staff. 6 August 2014.
<http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM262327.pdf>
- ¹⁷ FDA. *In Vitro* Companion Diagnostic Devices: Guidance for Industry and Food and Drug Administration Staff. 6 August 2014.
- ¹⁸ Turning the Tide Against Cancer. Sustaining Progress Against Cancer in an Era of Cost Containment. Discussion Paper. June 2012.
- ¹⁹ NORC at the University of Chicago. Evaluation of the Beacon Community Cooperative Agreement Program. Data Sharing to Enable Clinical Transformation at the Community Level: IT Takes a Village. Presented to ONC HIT. May 2014.
- ²⁰ PhRMA. Principles for Responsible Clinical Trial Data Sharing.
<http://www.phrma.org/sites/default/files/pdf/PhRMAPrinciplesForResponsibleClinicalTrialDataSharing.pdf>
- ²¹ Avalere Health. Lilly to Begin Sharing Clinical Trial Data with Researchers. *EBM Insights*. 9 June 2014.
http://ebm.avalerehealth.net/newsletters/194?article_id=903
- ²² Avalere Health. J&J Partners with Yale's Open Data Access Project in Clinical Trial Data Sharing Agreement. *EBM Insights*. 3 February 2014.
http://ebm.avalerehealth.net/newsletters/174?article_id=818
- ²³ Avalere Health. Milestone for Open Clinical Science with Medtronic Spine Study. *EBM Insights*. 24 June 2013.
http://ebm.avalerehealth.net/newsletters/144?article_id=688
- ²⁴ Avalere Health. FDA Advisory Panel Recommends Relaxing REMS for Avandia Based on Reexamined Data. *EBM Insights*. 17 June 2013.
http://ebm.avalerehealth.net/newsletters/143?article_id=683
- ²⁵ Avalere Health. GSK Launches Library of Clinical Trial Data to Enhance the R&D Process. *EBM Insights*. 22 October 2012.
http://ebm.avalerehealth.net/newsletters/113?article_id=543
- ²⁶ National Quality Forum. Report from the National Quality Forum: 2012 NQF Measure Gap Analysis. 31 March 2013. Funded by HHS.
http://www.qualityforum.org/Publications/2013/03/2012_NQF_Measure_Gap_Analysis.aspx
- ²⁷ Institute of Medicine. Delivering High-Quality Cancer Care: Charting a New Course for a System in Crisis. 10 September 2013.
http://iom.edu/-/media/Files/Report%20Files/2013/Quality-Cancer-Care/qualitycancercare_rb.pdf
- ²⁸ National Quality Forum. Patient-Reported Outcomes (PROs) in Performance Measurement. 10 January 2013.
www.qualityforum.org/Publications/2012/12/Patient-Reported_Outcomes_in_Performance_Measurement.aspx
- ²⁹ Robert Wood Johnson Foundation. ACA Implications for State Network Adequacy Standards. August 2013, <http://www.rwjf.org/en/research-publications/find-rwjf-research/2013/08/aca-implications-for-state-network-adequacy-standards.html>
- ³⁰ Pearson C. Consumers Face More Hurdles to Accessing Drugs in Exchange Plans Compared to Employer Coverage. Avalere Health. 14 March 2014. <http://avalere.com/expertise/life-sciences/insights/more-controls-on-drug-access-in-exchanges>
- ³¹ Abelson R. "More Insured, but the Choices Are Narrowing." *The New York Times*. 12 May 2014.
<http://www.nytimes.com/2014/05/13/business/more-insured-but-the-choices-are-narrowing.html>
- ³² National Committee for Quality Assurance. Patient-Centered Medical Home Recognition.
<http://www.ncqa.org/Programs/Recognition/Practices/PatientCenteredMedicalHomePCMH.aspx>

- ³³ Eagle D, Spradlio J. A Model for the Future: The Oncology Medical Home. *Practice & Policy, Survivorship, End-of-Life Care, Oncology Journal*. 13 June 2011. <http://www.cancernetwork.com/practice-policy/care-model-future-oncology-medical-home>
- ³⁴ Community Oncology Alliance. Accessed: http://www.communityoncology.org/pdfs/Clinical/Community_Oncology_2_0_Medical_Home_Report_CoC_OMH_Initial_Accreditation_Model.pdf
- ³⁵ Centers for Medicare and Medicaid Services. Health Care Innovation Awards Round One Project Profiles. December 2013. <http://innovation.cms.gov/Files/x/HCIA-Project-Profiles.pdf>
- ³⁶ Graham HR. Top Health Trend for 2014: Telehealth To Grow Over 50%. What Role for Regulation? *Forbes Magazine*. 28 December 2013. <http://www.forbes.com/sites/theapothecary/2013/12/28/top-health-trend-for-2014-telehealth-to-grow-over-50-what-role-for-regulation/>
- ³⁷ Fox S. Cancer 2.0: A Summary of Recent Research. December 2010. http://www.pewinternet.org/files/old-media/Files/Reports/2010/PIP_Cancer_20_Dec2010.pdfhttp://www.pewinternet.org/files/old-media/Files/Reports/2010/PIP_Cancer_20_Dec2010.pdf
- ³⁸ H.R.4302 – 113th Congress (2013-2014) Summary. Accessed: <https://beta.congress.gov/bill/113th-congress/house-bill/4302>
- ³⁹ UnitedHealth. Personalized Medicine: Trends and prospects for the new science of genetic testing and molecular diagnostics. Center for Health Reform & Modernization. Working Paper 7. March 2012. <http://www.unitedhealthgroup.com/~media/UHG/PDF/2012/UNH-Working-Paper-7.ashx>
- ⁴⁰ Meier DE and Brawley OW. Palliative Care and Quality of Life. *Journal of Clinical Oncology*. 2011. <http://jco.ascopubs.org/content/29/20/2750.full>
- ⁴¹ Centers for Medicare and Medicaid Services. Medicare Hospice Benefits. <http://www.medicare.gov/Pubs/pdf/02154.pdf>
- ⁴² Institute of Medicine. A Continuously Learning Health Care System in the United States. 12 July 2013. <http://www.iom.edu/Global/Perspectives/2013/ContinuouslyLearning.aspx>
- ⁴³ Panella M, Marchisio S, Stanislaw FD. Reducing clinical variations with clinical pathways. Do pathways Work? *Int J Qual Health Care* (2003) 15 (6): 509-521. <http://intqhc.oxfordjournals.org/content/15/6/509.full>
- ⁴⁴ Kinsman L, Rotter T, Snow P and Willis J. What is a clinical pathway? Development of a definition to inform the debate. *BMS Med*. 2010; 8: 31. <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2893088>
- ⁴⁵ Rotter T, Kinsman L, Machotta JEL, et al. Clinical pathways: effects on professional practice, patient outcomes, length of stay, and hospital cost (Review). *The Cochrane Collaboration*. 2010, Issue 7. <http://apps.who.int/whl/reviews/CD006632.pdf>
- ⁴⁶ The Cancer Policy Institute at the Cancer Support Community. Statement on Decision-making in Cancer Care. <http://www.cancersupportcommunity.org/General-Documents-Category/Policy/Statement-on-Decision-Making-in-Cancer-Care.pdf>
- ⁴⁷ Goldwater J, Yuhasz L. Considerations for Clinical Integration. Truven Analytics. December 2011. http://truvenhealth.com/portals/0/assets/HOSP_11363_0712_ClinicalIntegration_WP_Web_7662.pdf
- ⁴⁸ ONC-HIT Certification Program. <http://www.healthit.gov/policy-researchers-implementers/about-onc-hit-certification-program>
- ⁴⁹ Abernethy A, Abrahams E, Barker A et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clin Cancer Res* March 2014.
- ⁵⁰ Abernethy A, Etheredge LM, Ganz PA et al. Rapid-Learning System for Cancer Care. *J Clin Oncol*. 20 September 2010. <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2953977/>
- ⁵¹ Institute of Medicine. Assessing and Improving Value in Cancer Care. Workshop Summary. 4 November 2009. <http://www.iom.edu/Reports/2009/Assessing-Improving-Value-Cancer-Care.aspx>
- ⁵² American Society of Clinical Oncology. *ASCO Value Framework Fact Sheet*. July 28, 2014. http://www.asco.org/sites/www.asco.org/files/asco_value_fact_sheet_final_7_28_14.pdf
- ⁵³ Patient-Centered Outcomes Research Institute. National Priorities and Research Agenda. <http://www.pcori.org/research-we-support/priorities-agenda/>
- ⁵⁴ Avalere Health. Delivering Value in Healthcare: A Multi-Stakeholder Vision for Innovation. March 2013. http://www.avalerehealth.net/research/docs/031913_Dialogue_WhitePaper.pdf
- ⁵⁵ Agency for Healthcare Research and Quality. Communication and Dissemination Strategies To Facilitate the Use of Health and Health Care Evidence. July 31, 2012. Accessed at: http://effectivehealthcare.ahrq.gov/ehc/products/433/1208/CommunicationDissemination_ResearchProtocol_20120731.pdf
- ⁵⁶ Patient-Centered Outcomes Research Institute. PCORI Funding Awards. <http://pfaawards.pcori.org>