

Turning the Tide Against Cancer: Roundtable Proceedings

*Advancing Policy Solutions to Support Innovation in Cancer
Research and Care*

National Dialogue on Cancer Progress and Value

Washington, D.C.
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Overview

Cumulative advances in science and technology are yielding significant developments in oncology, with survivorship at an all-time high and patients living fuller lives following a diagnosis.¹ Emerging innovations are also creating new possibilities in the prevention, diagnosis, and treatment of the more than 200 diseases we collectively call cancer. In 2013, three new imaging technologies and 11 new treatments were FDA-approved for various types of cancer, nearly half of which were molecular-targeted agents.¹ A greater understanding of the molecular basis of disease, combined with ongoing efforts to apply these insights into clinical care, is forming the foundation of personalized cancer care.

Despite these advances, cancer remains the most costly disease in the United States and is predicted soon to become the number one disease-related killer as a result of changing demographics, rising obesity rates and tobacco use.¹ Without continued medical innovation to address this urgent clinical need and to improve patient outcomes, the economic burden of cancer is unsustainable. Nonetheless, the many scientific and technological advances that are redefining how we classify, diagnose, and treat cancer are being realized during a time of intense pressure for cost containment, and are at risk of being stifled.

The nation's challenge is to identify policy solutions that will preserve medical innovation that benefits each individual patient, while addressing the issue of escalating costs within the healthcare system overall. This challenge requires leadership commitment from a wide range of stakeholders willing to partner in support of solutions. In recognition of this need, the [American Association for Cancer Research](#) (AACR), the [Personalized Medicine Coalition](#) (PMC), and [Feinstein Kean Healthcare](#) (FKH) in 2012 launched a national dialogue on cancer progress and value. The dialogue began with a national conference, *Turning the Tide Against Cancer Through Sustained Medical Innovation*², and has continued since then, guided by an Advisory Committee of leaders from across the oncology community.

“The personalized medicine revolution will require revolutionary changes in how we care for cancer patients.” – J. Leonard Lichtenfeld, M.D., MACP, Deputy Chief Medical Officer, American Cancer Society

This national dialogue is focused on identifying policy solutions to achieve the following: sustain scientific and clinical progress; meet the challenge of rising healthcare costs with solutions that advance patient-centered, evidence-based, high-quality care; sustain adequate funding for scientific research in the public sector, accompanied by increased

¹ American Association for Cancer Research. AACR Cancer Progress Report 2013. *Clin Cancer Research* 2013;19(Supplement 1):S1-S88.

² For more information, visit: <http://turningthetideagainstcancer.org/>

efficiency in public and private sector research, and; provide incentives to accelerate progress against cancer, while simultaneously supporting efficient, affordable cancer care.

To seek potential policy solutions that align with scientific trends and patient-centered care, the co-conveners of the *Turning the Tide Against Cancer Through Sustained Medical Innovation* national conference advanced the policy dialogue by engaging leaders across sectors in a Roundtable discussion in October 2013. At the Roundtable, a group of 40+ participants representing the key stakeholder groups discussed the three core topics below (previously identified at the 2012 conference):

- **Cost and Value:** Demonstrating the value of personalized cancer care
- **Shifting to Patient-centered Care in Oncology:** Engaging patients in defining value
- **Continuous Learning:** Supporting a continuous learning healthcare system

Sustaining innovation in cancer research and care in an environment of intense pressure to contain rising healthcare costs requires new approaches and extensive innovation across all sectors in discovery and clinical research, and a research infrastructure and health policy framework that can support a rapidly changing cancer care ecosystem. In this report, we cover the diverse perspectives articulated in discussions at the Roundtable, and outline potential next steps for sustaining progress against cancer.

“Research that is necessary is not a cost, it is an investment.”

– John Nelson, M.D., M.P.H., Senior Advisor and FuturePanel Member, Leavitt Partners

Cost and Value

Demonstrating the value of personalized cancer care

“There are differences of opinion on what we mean by “cost” and “value.” And the reason that we have so many diverse opinions is that we don’t have the data, the data infrastructure, and the systems to support the generation of knowledge to help us truly measure and define what those pieces are.” – Amy Abernethy, M.D., Ph.D., Associate Professor of Medicine, Division of Medical Oncology, Department of Medicine, Duke University School of Medicine, Director, Duke Cancer Care Research Program

Incentives for progress and value can be aligned, but frequently they are not. Properly designed payment policy can recognize the ways innovation offers a solution for achieving high-quality, efficient healthcare. Many current policy proposals, however, tend to focus on narrower dimensions of value over shorter time horizons, and risk blunting continued progress.

It is important to understand the comparative clinical and economic value of the various dimensions of cancer care, but the current scientific, research, and care delivery ecosystem makes the effort to gain this understanding extraordinarily complex. Current approaches for assessing value are often limited in that they do not recognize and support the incremental nature of scientific and medical advances. Furthermore, static valuations at a single point in time do not capture the additional understanding we have of medicines as real-world knowledge and continued research reveal better ways to use many interventions. Current measures of value tend not to give adequate consideration to patient quality of life, patient preference, and indirect measures of value like productivity. New dynamic evidence generation capabilities are needed in order to provide accurate, patient-centered, and current assessments of economic and clinical value.

“I think one of the issues here is the tension between what is an individual cost and what is a societal cost. And I think that’s one of the most important things to discuss as we look at comparative effectiveness research, as we look at making decisions.”

– Gwen Darien, Director, The Pathways Project

Recognize patient sub-groups in value measurements. Current models of value measurement, such as comparative effectiveness research, measure population averages and thus do not take into consideration biological differences among patients and their tumors, which is the foundation of personalized medicine. This limitation suggests that more sophisticated approaches are needed that can handle the extreme heterogeneity of the diseases, as well as of the patients, in order to assess value at the *individual* level in addition to measuring value at the *population* level.

“When you add genotype to [inclusion criteria], every disease is an orphan disease.”
– William S. Dalton, M.D., Ph.D., Chief Executive Officer, M2Gen; Director, DeBartolo Family Personalized Medicine Institute, Moffitt Cancer Center

Incorporate broader measures of value into assessments. There are a variety of dimensions that constitute value, including improved overall survival, toxicity profile, and quality of life. While all dimensions of value must be taken into consideration, the patient’s concept of value should be given a primary role at the center of the cancer care ecosystem. Broader measures that are patient-centric, including quality of life, patient preference, and productivity should be incorporated into value assessments.

Measure the evolving value of treatments. It is generally true in medicine that patient outcomes improve over time as experience is gained with new treatments and interventions. Value assessment approaches must align with the way progress and knowledge evolves over time, which often reveals important information that increases the value or impact of many interventions. Evidence generation should include the ability to follow patients throughout their lifetime for the collection of observational, real-world data after clinical trials have ended. These new insights must then be incorporated back into the research and development process as well as patient care.

“We need better evidence to measure outcomes, especially in a heterogeneous world...but nobody is stepping forward to say they want to collect [and pay for] this evidence in aggregate. This is very limiting because it’s difficult to go back to insurance plans and say, ‘If you do the following, you can get better outcomes overall.’”

– Edward Abrahams, Ph.D., President, Personalized Medicine Coalition

Enable the development of clinical decision support tools. Clinical decision support tools should compare patient information with similar patients in a comprehensive database and facilitate conversion of these data into clinically useful knowledge. Policies that incentivize the establishment of a continuous learning healthcare system will facilitate the development of such tools.

“The question is really, when are the data good enough for clinical use and for reimbursement decisions? And I think that there is a need for more discussion and clarity around evidentiary standards.” – Sean Tunis, M.D., M.Sc., Founder, President, and CEO, Center for Medical Technology Policy

Develop regulatory policies to advance personalized medicine. The most expensive medicine is the one that doesn’t work. Strategies for the molecular stratification of patients, such as the development of companion diagnostics to guide treatment decisions, are one key element driving personalized, patient-centered oncology. Companion diagnostics hold substantial promise for improving patient outcomes and helping to control overall treatment costs. However, the absence of a rational approach for reimbursing the clinical use of companion diagnostics represents one significant challenge for the development of the molecular diagnostics pipeline.³ Research, regulatory, and reimbursement policies that will encourage innovators to develop these tools are greatly needed.

Additionally, policymakers and regulators must create an environment that enables new advances in patient care that are safe, accurate, and reliable; establishes a viable pathway towards patient access to new medical interventions; and, given the small patient sub-populations who qualify for clinical trials for molecular targeted agents, supports smaller trial sizes.

“Once we clarify what value is and agree upon it across the board, then we can look at how to generate outcomes data that are meaningful to patients.”

– Marcia Kean, M.B.A., Chairman, Strategic Initiatives, Feinstein Kean Healthcare

³ The Adverse Impact of the US Reimbursement System on the Development and Adoption of Personalized Medicine Diagnostics. The Personalized Medicine Coalition Website. [Cited August 6, 2013]; Available from: <http://www.personalizedmedicinecoalition.org/sites/default/files/files/Reimbursement%20Issue%20Brief%281%29.pdf>

“Policies are needed that support the development of companion diagnostics, because the most expensive therapy is the one that doesn’t work.”

– Edward Abrahams, Ph.D., President, Personalized Medicine Coalition

“Higher quality, lower cost products are delivered in every other sector of the economy, except healthcare. It’s an artificial market. And the reason is because we have no way, as providers of those services and products, to have our customer respond to and

reward innovation and efficiency.” – John J. Castellani, President and CEO,

Pharmaceutical Research and Manufacturers of America (PhRMA)

Shifting to Patient-centered Care in Oncology

Engaging patients in defining value

In this new era of personalized cancer care, there is a growing emphasis on defining the value of clinical interventions from a patient-centric perspective. Supporting the shift to patient-centered oncology will require engaging patients in more meaningful ways across the continuum of research and care, including developing more effective tools for measuring outcomes that matter to patients, and encouraging the creation of policy incentives for patient engagement and patient-provider decision support.

To ensure that the clinical needs of patients drive translational science and direct the development of meaningful outcomes measurements, patients must be engaged “where they are” (e.g., social networking communities, in the physician’s office) and asked directly about what they need and value throughout their individual patient journeys, from diagnosis through survivorship.

“The thing I wanted the most was not the latest chemotherapy. I was not worried about suffering. I wanted to appear before my grandchildren in a way that they would not be afraid ... Some wants for patients are quite different from what we think they are.”

– John Nelson, M.D., M.P.H., Senior Advisor and FuturePanel Member Leavitt Partners

Encourage patient participation throughout the research process. Improving patient outcomes is a collaborative endeavor between the patient and clinical research communities; however, only five percent of cancer patients participate in clinical research.⁴ Enhancing the value proposition for patients could improve enrollment. For example, Moffitt Cancer Center’s Total Cancer Care® has successfully collected tissue, data, and consent from more than 50,000 patients to date by simply giving the patients something in return: their data. Policies that promote patient engagement throughout the research process should be considered.

⁴ American Association for Cancer Research. AACR Cancer Progress Report 2013. *Clin Cancer Research* 2013;19(Supplement 1):S1-S88.

***“The most consistent complaint that I hear from patients is: ‘What’s happening to me?’
And we’re not telling them what to expect.” – Douglas Moeller, M.D., Medical Director,
McKesson Health Solutions***

Support shared decision-making. Patients can become more involved in their medical care through shared decision-making (evidence-based decisions at the individual level) with their oncologists. Physicians, patients, and caregivers should be supported in shared decision-making through the development and use of clinical decision support tools (e.g., Archimedes IndiGO) that are designed to elicit and capture patient input and support patient-physician engagement. Additionally, providers must be encouraged to have conversations with patients on the costs of care so their patients can make the most informed decisions.

***“It’s very hard to put labels on what patient-centeredness is because it’s a very different thing to different people.” – Ellen Sigal, Ph.D., Founder and Chairperson,
Friends of Cancer Research***

Empower patients through education. A cancer diagnosis can be one of the most terrifying events in a patient’s life. Although the Internet is flooded with information — and misinformation — about cancer, most patients do not know where to go for information that is *relevant to their specific disease*. Physicians must be able to guide their patients to evidence-based data that are relevant to their disease or disease subtype, either through a patient portal or online library. Making such data easily accessible will empower many patients to become more involved in their clinical care.

Continuous Learning

Supporting a continuous learning healthcare system

“I think that there is an enormous cost to have completely separate systems, one for clinical care and one for clinical research. The research communities are paying fortunes to try to get access to clinical data, which is not well organized to serve those purposes, and in clinical care we have the inability to learn as we go.”

***– Laura Esserman, M.D, M.B.A., Professor of Surgery and Radiology, University of California, San Francisco, Director, Carol Franc Buck Breast Care Center, Co-Leader, Breast Oncology Program,
UCSF Helen Diller Family Comprehensive Cancer Center***

The systems of clinical research and healthcare delivery operate as separate entities, or silos. This separation is a costly endeavor and an ineffective use of resources in that it deprives clinicians of evidence-based research to inform decision-making, patients of the expected best clinical care, and policymakers of a rational basis for choosing the most effective policies.⁵ Policies that promote the integration of clinical research and care into

⁵ IOM. 2012. *The Clinical Trials Enterprise in the United States: A Call for Disruptive Innovation: Discussion Paper*. Washington, DC: The National Academies Press.

one seamless continuum are needed in order to facilitate system-wide learning and leverage the clinical experience of all cancer patients, while simultaneously helping to reduce the cost and accelerate the process of drug development.

“The one big change I’d like to see is that we get a shared understanding of clinical evidence and how to evaluate evidence to guide clinical decisions. I think that can help us develop a better agenda for comparative effectiveness research and other types of research that could lead to better use of technology to try to help patients with cancer.”

– David Hickam, M.D., M.P.H., Program Director, Clinical Effectiveness Research, Patient-Centered Outcomes Research Institute (PCORI)

Develop a health IT infrastructure. A learning healthcare system requires a robust health information technology (IT) infrastructure that will seamlessly link research and care and enable the continual, automatic collection and compilation of data from clinical practice, disease registries, clinical trials, and other sources of information. This system should facilitate the use of these data to deliver the best, most up-to-date care that is personalized for each patient.⁶

Develop data standards and methods for data collection and aggregation. Within the health IT infrastructure, solutions must be considered that allow for appropriate access to data for research purposes while protecting patient privacy. There must also be assurance that analyses are methodologically rigorous, and that appropriate standards are in place for the communication of accurate research findings by entities in the public and private sectors.

Incentivize data sharing. Cancer centers should be incentivized to share and access data across networks. A core data set that enables learning from every patient should be collected from every patient throughout his/her journey, from diagnosis through survivorship. The core data set would include clinical data, genomic and molecular data of patient and tumor, as well as patient-reported outcomes. These data can serve as the foundational component of a clinical decision support tool.

“Pay for data, not interventions.” –*Laura Esserman, M.D, M.B.A., Professor of Surgery and Radiology, University of California, San Francisco, Director, Carol Franc Buck Breast Care Center, Co-Leader, Breast Oncology Program, UCSF Helen Diller Family Comprehensive Cancer Center*

Optimize data privacy policies. To realize the potential of this infrastructure requires solutions that allow for appropriate access to data for research purposes while protecting patient privacy.

⁶ IOM. 2010. *A foundation for evidence-driven practice: A rapid learning system for cancer care: Workshop summary*. Washington, DC: The National Academies Press.

“All the data that we’re collecting is great, but data in itself is not information. We need evidence. We need to take that data and turn it into real evidence and it doesn’t matter to me whether it comes from clinical trials or comparative effectiveness research or a learning healthcare system. We need to make sure that what we’re doing with that data actually gets us to interventions for patients.” – Sharyl Nass, Ph.D., Director,
National Cancer Policy Forum, Institute of Medicine

“What we need are mechanisms to collect much more robust clinical data in a doable way and to have that data be shareable, interoperable, liquid.”

– Lawrence N. Shulman, M.D., Chief Medical Officer, Senior Vice President for Medical Affairs,
Dana-Farber Cancer Institute and Associate Professor of Medicine, Harvard Medical School

Facilitate interoperability. The interoperability of electronic health records (EHR) will enable data transfer across EHR networks throughout the healthcare ecosystem. Solutions are needed that facilitate standards-based interoperability between electronic health record systems as well as incentivize the incorporation of outcomes data in EHRs.

Build a comprehensive clinical database

The exchange of high-quality clinical data across institutions is greatly needed; this sharing must include the genomic and molecular data of the patient and his/her tumor, as well as patient-reported outcomes. Patients can retain control of their data by self-nominating who can have access to their biospecimens and clinical data (e.g., basic research, clinical trials). A comprehensive database can facilitate our understanding of how the molecular basis of disease changes over time and if interventions are working for patients with a particular disease or molecular subgroup.

Incentivize Collaboration

A silo mentality in which key players in cancer research and care think only in terms of their own sector or institution is incompatible with scientific progress. A highly collaborative multidisciplinary ecosystem is needed in which all stakeholders — industry, academia, government, clinicians, patients, and advocates — work towards a common vision to translate scientific discoveries into better patient care. New models of collaboration require a cultural shift as well as incentives for data sharing. Tax policies and/or federal funding are vital for incentivizing collaboration and data sharing across institutions.

“We need to become better partners, and we are making headway in this endeavor.”

– Zeba Khan, Ph.D., Vice President of Global Strategic Market Access & Policy,
Celgene Corporation

Building the Foundation to Promote Meaningful Change

Before meaningful change can be realized throughout the research and clinical communities, two foundational elements must first be in place: governance and honest dialogue.

“Without trust, there’s nothing that we can do. We can’t share data. We can’t collaborate. We can’t engage patients.” – Kathleen Foley, Ph.D., Senior Director, Strategic Consulting, Truven Health Analytics

Governance. If the vision of personalized cancer care is to be realized, high-level leadership committed to defining policy approaches that incentivize biomedical innovation is needed. Meaningful change cannot occur without commitment, multi-stakeholder engagement, and strong leadership.

Honest dialogue. Research and innovation are an investment in the future of American health and must be sustained; however, an honest dialogue is needed about the costs of care. For example, innovation by itself will not always reduce healthcare costs (e.g., cost of care may increase as patients live longer). Additionally, the economic component of value, and the controversial issues this topic presents (e.g., cost limits per patient, end-of-life care, societal versus individual needs and wants), is an important aspect that cannot be overlooked in policy discussions. All members of society must be engaged in these critical discussions.

Proposed Next Steps

“Innovation is key to more cost effective solutions.” – William S. Dalton, M.D., Ph.D., Chief Executive Officer, M2Gen; Director, DeBartolo Family Personalized Medicine Institute, Moffitt Cancer Center

Innovation is an investment in the health and wellness of the American people, and is necessary to ensure that cancer care is personalized, patient-centered, evidence-based, and efficient. To sustain progress against cancer in an era of cost containment requires commitment, multi-stakeholder engagement, strong leadership, and a flexible policy framework.

Given the broad array of potential issues to be addressed for the betterment of cancer patients, Turning the Tide Against Cancer Through Sustained Medical Innovation Roundtable participants were in agreement that a practical, larger framework needs to be developed for moving forward. This framework will outline new approaches for sustaining innovation in oncology care, with the following three areas identified as key to developing a path forward:

- 1) **Encourage the development and adoption of patient-centered quality measures.** Engage patients in the process of developing value and quality measurements that more closely align with their needs and preferences, and scientific advances.
- 2) **Enable continuous learning to optimize shared decision-making between patients and their physicians.** When appropriately used, emerging health IT and EHR capabilities have the potential to enable the collection and analysis of real-world data, and capture emerging advances in the science. These technologies will provide patients and physicians a valuable tool as they work through a care plan.
- 3) **Encourage meaningful patient engagement through greater collaboration and partnerships.** Two-directional communication and sharing, on both an individual patient-physician level and also between larger entities, is integral to continued advances in cancer care. Collaboration is also key in developing standards and incentives for data collection and information exchange.

Advancing Innovation

The Turning the Tide Against Cancer initiative continues to work towards solutions that will ensure policy and regulatory alignment with advancing scientific opportunities. In 2014, the initiative co-conveners will once again engage stakeholders and experts within the cancer community in the ongoing dialogue about sustaining innovation, frame specific policy solutions, and highlight the value of scientific progress to policymakers. A series of expert panels will explore actionable policy solutions related to patient-centeredness, learning healthcare systems, and cost and value. A conference in October 2014 will provide a forum to share the findings from these panels and continue to advance the discussion about policies that will enable sustained progress against cancer in a cost-constrained environment.