

The Personalized Medicine Coalition, representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

Cancer Conference Highlights Policies Needed to Sustain Biomedical Innovation

The growing health needs of an aging population and the desire to rein in government spending challenge policymakers in Washington, D.C. and indeed around the world. The high stakes for personalized medicine motivated the Personalized Medicine Coalition, **American Association for Cancer Research and Feinstein Kean Healthcare** to convene *Turning the Tide Against Cancer Through Sustained Medical Innovation* to bring together leaders in the cancer community for a discussion about how to define value; ways to address the need for health care cost containment; and opportunities to put in place public

policies necessary to promote innovation in cancer research and care.

The multi-stakeholder conference, held June 12 in Washington, DC, recognized recent scientific advances in preventing, detecting, diagnosing and treating cancer. It brought together leaders from business, science, and government and patients to focus on the need for better-designed public policies to motivate institutions to continue to push the envelope of innovation.

Keynote presentations from John Mendelsohn, M.D. of MD Anderson Cancer Center, Mark McClellan, M.D., Ph.D. of the Brookings Institution, and

Siddhartha Mukherjee, M.D., author of *The Emperor of All Maladies*, framed the discussion and provided further insights on cancer science and policy.

“We’re going to get to an era of more personalized medicine. The only question is how long it’s going to take, how hard it’s going to be, and how much else we’re going to have to give up in our society in order to accomplish that goal,” said McClellan.

“We sought to identify the policy opportunities that can bridge the gap between the science and the patient,” noted Edward Abrahams, Ph.D., president of the Personalized Medicine Coalition.

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FROM THE PRESIDENT



Personalized Medicine Coalition Takes Stock

BY EDWARD ABRAHAMS

Each fall many people take stock of their lives, and ask if they are on the right track. Looking ahead, organizations, especially membership organizations like the Personalized Medicine Coalition, also engage in a process of self-evaluation, though we call it strategic planning.

As the fall PMC newsletter demonstrates, this has been a busy time for the Coalition—in education, advocacy,

and creating networking opportunities for our members to share best practices and to learn from one another, a rare opportunity in health care since PMC’s members cover so many different sectors.

Our conference, *Turning the Tide Against Cancer Through Sustained Medical Innovation*, articulated how and why public policy is so critical for the future of cancer research and clinical care.

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POLICY UPDATE

FDA User Fee Reauthorization Signed Into Law

On July 9, President Obama signed into law bipartisan legislation to reauthorize the user fees that industry must pay to the FDA for the review of new products. Provisions of the new law directly related to personalized medicine include:

- The development of FDA's capacity to review submissions involving pharmacogenomics and biomarkers by integrating and training staff with pharmacogenomics and biomarker expertise into the product review process.
- The creation of an expedited approval process for "breakthrough therapies"—drugs that are both developed using biomarkers and used to treat serious or life-threatening ailments.
- A requirement that the FDA notify Congress two months in advance of the Agency's planned release of any guidance or draft guidance document related to the regulation of laboratory-developed tests.

Stephen Spielberg Discusses FDA's Personalized Medicine Activities

In an address to the Personalized Medicine Coalition (PMC) Policy Committee on July 18, Stephen Spielberg, M.D., Ph.D., Deputy Commissioner for Medical Products and Tobacco at the U.S. Food and Drug Administration (FDA), announced that the Agency will develop a catalog of personalized medicine-related activities. The catalog will provide a full accounting of

the agency's relevant activities, including all regulatory divisions and regulatory science initiatives.

Dr. Spielberg discussed the opportunity to advance personalized medicine through communication among stakeholders and FDA Centers. "PMC is so important because we need dialogue; no one has a lock on complete information," he told the committee.

Federal Circuit Court Again Upholds Patent-Eligibility of Myriad's Isolated DNA Claims, Holds Diagnostic "Analyzing" Claims Patent-Ineligible

On August 16, the U.S. Court of Appeals for the Federal Circuit (CAFC) held that Myriad's composition claims regarding "isolated" DNA molecules concern subject matter that can be patented under 35 USC §101. The CAFC also upheld the drug screening claim on growing host cells transformed with an altered *BRCA1* gene in the presence or absence of a potential cancer therapeutic. The CAFC ruled that the Myriad method claims "analyzing and comparing certain DNA sequences" were patent ineligible. The ruling came after the Supreme Court issued its decision March 20, 2012 in *Prometheus v. Mayo*, which had invalidated certain Prometheus Laboratories' method claims and vacated the July 29, 2011 decision in the Myriad case. The case has now been appealed to the Supreme Court with intellectual property implications for personalized medicine.

8TH ANNUAL PERSONALIZED MEDICINE CONFERENCE

NOVEMBER 28-29, 2012

JOSEPH B. MARTIN CONFERENCE CENTER
AT HARVARD MEDICAL SCHOOL, BOSTON

This two-day conference will focus attention on some of the exciting accomplishments and some of the pressing challenges that bear on Personalized Medicine. Distinguished speakers and panelists will consider how recent developments and experiences may guide and inform the policies, plans and actions of stakeholders among government, healthcare providers, academe and the private sector. Conference sessions will include discussions on technological, business, legal, policy and ethical issues as well as attention to what is happening internationally.

The conference also provides ample opportunities for networking and establishing new relationships.

Online registration and conference details can be found at: WWW.PERSONALIZEDMEDICINECONFERENCE.ORG

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The conference kicks off on November 27 with a PMC cocktail reception at the Commonwealth Hotel, 500 Commonwealth Ave, Boston, MA., 6-9 p.m. Please register at bitly.com/TNIRTP.



Affordable Care Act Implementation: Is PCORI Living Up to Its Promise?

BY AMY MILLER | VICE PRESIDENT, PUBLIC POLICY

During this election season, there has been a lot of talk about the Affordable Care Act and what the implementation of it means to our nation and patient care.

For personalized medicine, that means watching the Patient-Centered Outcomes Research Institute (PCORI) begin to develop its infrastructure and define its policies. PCORI was created to provide a scientific rationale for the selection of medical treatments with an eye towards finding inefficiencies in the health care system. In a speech outlining his thinking, President Obama talked about comparing red and blue pills. Why, he argued, should we pay for the expensive blue pill, if the less expensive red pill works just as well? But the President's catchy political rhetoric ignored the reality that red pills and blue pills may not work the same for everyone.

A bipartisan group of six senators eventually came to understand that our nation did indeed need good, real-world evidence that compared medical treatments and sought community input regarding the structure of the legislation that authorized PCORI.

Congress wanted to design something new and different. No longer satisfied with spending billions of federal dollars on science that more or less stayed in the ivory tower or took 17 years to reach clinical practice, these legislators wanted researchers to engage with the rest of the health care community to solve real-world problems—problems perhaps too complicated and confounding for traditional academic research.

Also, recognizing the limitations of our nation's current research enterprise, they designed the statute to incorporate public

engagement features and put in place a mandate to keep the research current.

Legislative drafters wanted the focus of comparative effectiveness research to be on the patient, to ensure it incorporated their values and desired outcomes. They understood that science is rapidly advancing and our nation's investment in new information should not ignore the federal investment already made in sequencing the human genome. Thus the statute directs PCORI to engage patients and the public, develop a system for updating the information they publish and directs the methodology committee to incorporate molecular and genetic subtyping into its work.

We are concerned that implementation is not adhering to legislative intent. For example, while PCORI contemplates funding new science, it has not articulated how it plans to keep that science updated once findings are released for public consumption, nor has it articulated how it will distribute the health information developed from the funded research. Also, while there is a lot of activity related to public engagement, it seems superficial. For example, PCORI provides little insight into how public comments are incorporated into their work.

PCORI has the opportunity to live up to expectations and achieve the goals outlined in statute. This summer, the methodology committee released a draft methodology report that it plans to re-draft based on public comments. Designed to be a living document, the draft, unfortunately, ignores personalized medicine. PMC,

like many in the community, submitted significant suggestions. In our letter we:

- Urged PCORI to follow statute and recognize personalized medicine by either developing a separate section on standards for use of biomarker and genetic variables in research or adding an additional section on heterogeneity of treatment effectiveness to be developed to guide the use of biomarker data.
- Encouraged it to embrace the opportunity to explore alternative methods for patient-centered outcomes research—including methodological standards for different types of real-world studies and appropriate statistical methods to study them.

Many PMC members such as the **American Association for Cancer Research**, the **American Clinical Laboratory Association**, the **American Medical Association**, the **Association for Molecular Pathology**, the **Biotechnology Industry Organization**, the **College of American Pathologists**, and the **Pharmaceutical Research and Manufacturers of America**, also submitted substantive comments to PCORI, which emphasized the importance of personalized medicine. Those comments and all others can be accessed at www.PCORI.org.

We are in danger of funding more research outlining what works best for most, research that ignores the principles of individual validation and would lock our health care system in the inefficient one-size-fits-all model that Congress wanted to move beyond when it established PCORI.

Conference Highlights Policies to Sustain Biomedical Innovation

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“Smarter regulations to encourage targeted therapeutics, more appropriate payment policies, and a government-wide commitment to advancing personalized medicine are needed to translate our emerging understanding of the causes of cancer into treatments that improve outcomes and lower overall health care costs,” he added.

Setting the stage for the conference, Laura Esserman, M.D., co-leader, breast oncology program, UCSF Helen Diller Family Comprehensive Cancer Center and Kathy Giusti, president and CEO, **Multiple Myeloma Research Foundation (MMRF)** along with other panelists, pointed out how innovative research models have been successful in improving the efficiency of cancer clinical trials by incorporating the principles of personalized medicine.

“Every single compound [Novartis Oncology has] in exploratory

development has a patient selection marker associated with it,” explained Christi Shaw, MBA, executive vice president at Novartis Oncology.

But, they also agreed that with the availability of information comes a necessity to find the means to biobank tissue samples and share data necessary to find new treatments that will work for patient subgroups.

Nevertheless, as patient populations become smaller, questions about the cost of new innovations have surfaced because

“We sought to identify the policy opportunities that can bridge the gap between the science and the patient.”

—Edward Abrahams, Ph.D., president of the Personalized Medicine Coalition

the per-capita costs of drug development have risen. In an era of cost containment, policymakers are considering the value of proposed treatments, sparking discussions about whether new medicines and medical research are worthwhile investments.

A second panel examined the question of value in more depth, but it agreed that value is ultimately defined by the patient and his or her needs and desires.

“Facing advanced disease, some cancer patients will choose aggressive chemotherapy regimens while others will opt for palliative care approaches that seek to maximize quality of life without treating the cancer itself,” Gwen Darien, director of The Pathways Project reminded the panel.

Patient values are definitely part of the equation, but so is cost, added Lee Newcomer, M.D., Senior Vice President for oncology at United Healthcare, recalling a story of a 25-year-old patient with stage IV colon cancer who chose to go on a canoe trip rather than immediately enrolling in a clinical trial once he realized that medicines might extend his life but would not cure his disease. “We [as a society] have got to have the discussion about what we can afford to pay,” he said, echoing a statement made by William Dalton, M.D., Ph.D., director of the **H. Lee Moffitt Cancer Center**, that physicians have a responsibility to educate their patients about what outcomes are realistic.

Panelists, however, saw personalized medicine as a way to increase value in the health care system. “While we can’t afford everything, we must get the right therapies to the right patients, and give the patient meaningful amounts of time and meaningful quality of time,” said Richard Gaynor, M.D., vice president for Clinical Development at **Eli Lilly**. The panel agreed that the discussion of value should be informed by patient data as well as by patient preferences.



MAX TAYLOR PHOTOGRAPHY

Siddhartha Mukherjee, M.D., Pulitzer Prize-winning author of *The Emperor of All Maladies: A Biography of Cancer*



MAX TAYLOR PHOTOGRAPHY

John Mendelsohn, M.D., Director, Khalifa Institute for Personalized Cancer Therapy, The University of Texas MD Anderson Cancer Center

“When you start generating evidence of what’s actually working for whom, we probably don’t need [policymakers to decide what constitutes value] because we’ll have evidence of what’s working,” concluded Dalton.

The need for better methods for evidence generation and data collection were common themes across panels and speakers. During a provocative lunchtime keynote, Mukherjee contended that we need a dramatic transformation in the platforms of drug discovery to replace the National Institutes of Health grant structures,

“The whole community is responsible for aligning the systems to get where the biology is heading.”

—Amy Abernethy, M.D., Director of the Duke University Cancer Care Research Program

which, he said, were not conducive to the discovery of new medicines. He also called for greater collaboration among government, industry, and academe in speeding clinical trials, expanding patient participation, and evaluating more effectively the quality of data.

While John Castellani, CEO of PhRMA, started the conversation with the upbeat news that PhRMA’s just-released “Medicines in Development” report found there to be more than 1,000 cancer medicines in pharmaceutical company pipelines, panelists identified the funding of innovation, as well as the cost of evidence generation (especially for diagnostic tests), as a deterrent to progress.

“There is no business model [for diagnostics companies],” lamented David Parkinson, M.D., of the venture firm New Enterprise Associates. “Where the rubber meets the road is who is going to pay for the development of these tests?”

Despite Parkinson’s pessimism,

Kenneth Anderson, M.D., of Dana Farber Cancer Institute remained optimistic: “I have a lot of faith that if we have innovation, [society] will figure out how to pay for it,” even as other panelists exclaimed that there are no guarantees that even FDA-approved companion diagnostics could win reimbursement from public and private payers.

“The whole community is responsible for aligning the systems to get where the biology is heading,” concluded Amy Abernethy, M.D., Director of the **Duke University** Cancer Care Research Program wrapping up the conference.

The conference was informed by a discussion paper “Sustaining Progress Against Cancer in an Era of Cost Containment,” based on interviews with more than 30 experts and thought leaders in the cancer community. The paper, along with videos from the conference, can be accessed at www.turningthetideagainstcancer.org.



MAX TAYLOR PHOTOGRAPHY

Christi Shaw, Executive Vice President and North America Region Head, Oncology at Novartis

Global Laboratories for Developing Personalized Medicine

In February, the Canadian government announced a \$67.5 million investment “that will help Canadians get more effective treatments and make the health care system more sustainable through personalized medicine.” The United Kingdom (UK) reported in April that its Stratified Medicine Programme is well on its way to collecting genetic data on 9,000 cancer patients to test the feasibility of implementing personalized medicine throughout the National Health Service. And, France continues to expand the list of genetic tests freely available to all of its citizens undergoing diagnosis and treatment for cancer.

These moves by national governments signify not just a rising tide of interest in personalized medicine, but a growing recognition that in order to succeed, personalized medicine needs a supporting infrastructure and government backing to build it.

To date, no country has implemented a system-wide strategy that fully facilitates the development and adoption of personalized medicine from research to the clinic. But selected countries around the world have become policy laboratories, building segments of an infrastructure to support the paradigm, including in research, health care delivery, regulation, and payment.

European Countries Beginning to Develop Mechanisms to Support Personalized Medicine

At least two studies released in the last year suggest that Europe is leading the way in putting in place the institutions which will support personalized medicine.

According to “Quantifying Utilization of Personalized Medicine Therapeutics,” a study by Sean Hu, Ph.D., of BioNest, and colleagues at MIT, Harvard and IMS Health, a total of 27 drugs have been identified as personalized therapeutics in use worldwide—those approved by regulatory authorities or recommended by professional societies to be used in conjunction with a

diagnostic test. Looking at the utilization of these drugs, the study found that Europe, Japan and other countries are now surpassing the United States (US) in per capita usage of personalized medicine.

A white paper, “Advancing Access to Personalized Medicine: A Comparative



The Honourable Leona Aglukkaq, Minister of Health, Canada

Assessment of European Reimbursement Systems,” written by Susan Garfield of **Bridgehead International** and published in October 2011 by the Personalized Medicine Coalition, looked at reimbursement policy in the European Union (EU). It concluded that Germany, France and the UK led the region in creating a more welcome environment for introducing personalized medicine products.

“In a lot of European countries, genetic testing has been going on for a

long time; a surprisingly high number of genetic tests are available,” says former PMC Board Member Robert Wells, now Head of the Biotechnology Unit at the Organization of Economic Cooperation and Development (OECD) in Paris. “In addition, its leadership rests on the fact that they are all single payer systems, which allows a level of integration that can promote a new drug or diagnostic throughout the country.”

But while centralized health care systems, such as those in Europe, can adopt and pay for personalized medicine products and services nationwide, payment decisions are still often left to regional and local jurisdictions. Consequently, coverage of drugs and companion tests can be inconsistent. “We literally had to go country by country, hamlet by hamlet, village by village, trying to get an understanding of who orders the test and what’s their decision process?” noted Harry Glorikian, whose life science strategy firm **Scientia Advisors** helps many companies navigate the global personalized medicine market. He said that challenges still exist for personalized medicine innovators in Europe and around the world despite governmental investments in setting up the infrastructure needed to bring personalized medicine into full use.

France Builds Framework for Adoption of Cancer Diagnostics

France has placed personalized medicine toward the top of its national health care agenda, particularly for cancer. With its

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—Harry Glorikian, Founder and Managing Partner, Scientia Advisors

2009 National Cancer Plan, France's Institut National Du Cancer (INCa) has put in place mechanisms to support the use of personalized medicine in cancer treatment, including full, universal coverage for several key molecular diagnostic tests.

A network of 28 regional labs throughout France offer diagnostic tests. They are provided free of charge to both patients and hospitals. Currently, coverage includes about six tests on some of the more common cancers (breast, colorectal, gastric, lung and blood). INCa recently reported that about nine other molecular diagnostic tests are being reviewed for potential coverage.

"Rapid access to innovation," says Dr. Fabien Calvo, Deputy Director General of the INCa, in order "to offer each patient in France equal access to medical tests as soon as new targeted therapies become available."

The French health care system has enabled it to achieve that goal. Around the middle of 2008, the EMA approved Erbitux and Vectibix for colorectal cancer patients. The drugs are most effective against tumors that do not have the KRAS mutation. By the end of 2008, the INCa provided funding for KRAS tests, and by the end of 2009, the tests were administered to all 18,000 colorectal cancer patients that incur metastatic disease in France each year.

Comparing the cost of subsidized testing to the savings realized through improved treatment, the French government calculates that its investment in personalized medicine is worthwhile, although the equation may not be so favorable from the diagnostic company perspective. "France has a great network for distribution, but they pay well below the value of the test," says one executive from a diagnostic company trying to expand its multiplex test into Europe.



Dr. Fabien Calvo, Deputy Director General of the Institut National Du Cancer, France

United Kingdom Tests New Cancer Products; Limits Use of Products Not Deemed Economically Beneficial

Beginning with a mechanism to collect and assess cancer tissue samples, the United Kingdom is also developing a personalized medicine infrastructure. But while the UK sees potential for personalized cancer medicine, it lacks the infrastructure to facilitate quick adoption of products that pass the UK's tests for economic benefit, which can be a barrier.

In the UK, national health agencies are collaborating on a Stratified Medicine Innovation Platform. Launched in July 2011, the platform supports a £200M five-year program to accelerate the development of personalized medicine through research support and its adoption in the clinical setting.

Towards the latter goal, Cancer Research UK and two pharmaceutical companies started a demonstrator project to test around 9,000 patients newly diagnosed with one of six tumor types (breast, gastrointestinal, lung, prostate, ovary and melanoma) for a number of common gene mutations. They want to

establish a framework to collect and secure samples and molecular data, making them accessible to both researchers and clinicians. Ultimately, their goal is to routinely test *all* NHS cancer patients nationwide for genetic mutations relevant to treatment.

"In the future, we anticipate that there will be hundreds if not thousands of tests that would need to be done for each patient, to make sure that the therapy chosen is exactly the one [from which] the patient would have the most benefit," said David de Castro, Ph.D., Head of Molecular Diagnostics at The Institute of Cancer Research.

But while the UK explores new mechanisms to identify the optimal therapy for a given cancer patient, the country curtails the use of products that don't pass economic muster with the National Institute for Health and Clinical Excellence (NICE), which evaluates all medicines and tests for economic benefit before they can be used broadly with patients. In an effort to better understand the economic impact of personalized medicine, last year NICE established a program to assess the cost-effectiveness of personalized medicine diagnostics and paired therapeutics.

The agency's decision in February not to recommend three genomic diagnostic tests designed to guide treatment for breast cancer (Oncotype DX, MammaPrint, Mammostrat) signaled that personalized medicine products could face challenges. NICE based its assessment on the contention that the tests have insufficient evidence to demonstrate clinical and economic effectiveness. Since then, more evidence has been presented and the decision is under review.

Luxembourg Partners with Institute for Systems Biology to Further Systems Medicine

The Grand Duchy of Luxembourg has seen not only the opportunity to transform health through personalized medicine,

but also the chance to diversify and strengthen its economy. It has initiated a historic five-year, \$100 million partnership with the **Institute for Systems Biology (ISB)** in 2008 to fund innovative science initiatives in P4 Medicine (predictive, preventive, personalized and participatory) to accelerate translation of new knowledge into novel diagnostic, therapeutic and prevention strategies, while moving the Luxembourg economy away from the financial services sector.

“Partnerships with academia, industry, foundations and national governments allow us to bring some of the world’s best scientists together to attack big and challenging problems of systems medicine,” said ISB President Leroy Hood, M.D. Ph.D., who conceived of and leads the project. “More countries must follow in Luxembourg’s footsteps to create a global laboratory for P4 Medicine where we can assemble complementary scientific and engineering expertise, develop new technologies, access data and specimens, promote joint fundraising efforts—and as a result, accelerate translation of new knowledge into novel diagnostic, therapeutic and prevention strategies.”

Rapid Adoption of Personalized Medicine Gives Israel Strategic Advantage

Israel’s ability to bring personalized medicine to patients, like France’s, is aided by its centrally-organized health care system which allows for quick adoption of new personalized medicine products. Israeli patients benefit from rapid adoption of personalized medicine breakthroughs and from a health information technology that is already fully-integrated into the delivery system.

In Israel, all individuals’ health care costs are covered by one of four major non-profit HMOs overseen and funded by the government. Each HMO must provide a comprehensive “services basket” which is reviewed and updated at least once each year. It includes services ranging from

“Israel tends to move quickly on technology. The translational period is very short between the publication of clinical evidence and clinical practice.”

—Gadi Rennert, M.D., Ph.D, Director of National Cancer Control Center, Clalit Health Services

preventive care to heart transplantation. According to Gadi Rennert, director of National Cancer Control Center for Israel’s largest HMO, Clalit Health Services, “Israel tends to move quickly on technology. The translational period is very short between the publication of clinical evidence and clinical practice.”

For example, after ASCO presentations in June 2011 validated ALK testing for treatment of lung cancer, and BRAF testing for treatment of melanoma, the tests and drugs described entered Israel’s services basket in January 2012. “We are probably one of the first countries in the world to provide these personalized treatments to the whole population within 5-6 months of the publication of favorable evidence,” said Rennert.

Aware of its opportunity to fully integrate personalized medicine into its health system, Israel’s National Institute for Health Policy Research, recently held a workshop at the Dead Sea to consider its policy options and will soon issue a white paper on personalized medicine opportunities.

Canada Invests in Research But has not Adopted Strategic Plan to Develop Personalized Medicine

While Canada is investing heavily in personalized medicine research, and has set up Genome Canada and regional

Genomics-focused institutes to fund research projects, it has yet to establish a health-focused institution to advance personalized medicine more rapidly. According to Katherine Bonter, Director of Advocacy at the Center of Excellence in Personalized Medicine (**CEPMED**), there is no national system for developing reimbursement codes and policy. Instead, those decisions are often made within individual hospital budgets. Bonter calculates that it can take two years to develop a reimbursement code for a new diagnostic test within a single province. She and her CEPMED colleagues point out that “Canadian tax payers have invested over a billion dollars in medical research related to genetics and biomarkers, but that this new knowledge has not been successfully translated into economic activity.”

When panitumab and cetuximab were approved in 2008 for colorectal cancer, testing for the KRAS mutation was recommended to prescribe the drugs, but payment remained unresolved. In this case the drug manufacturers (**Amgen** and **Bristol-Myers Squibb**) stepped in to pay for the tests so their drugs could be prescribed.

Despite these hurdles, Bonter, along with many others, remain optimistic. “I think we actually have a huge strength that people are starting to recognize. Each of the provincial health care systems is quite large and has access to the kind of data that would facilitate management of personalized medicine. If they can decide that something is worth implementing, it can potentially be implemented quickly throughout the system.” The key elements needed to realize that potential, she says, are the harmonization of Laboratory Developed Test (LDT) regulation, a more systematic approach to reimbursement, and a concerted effort to educate physicians on the clinical use and benefits of personalized medicine so that the large investments the country is making are translated into better medicine.

How will Japan Facilitate Growing Use of Personalized Medicine?

In Japan, the per capita use of personalized medicine tests and treatments has pulled ahead of that in the United States, in part due to the centralized nature of the Japanese health care system, which grants universal access to products once they are approved. Nevertheless, public announcements of new policies and government initiatives intended to encourage personalized medicine have yet to occur.

Although the regulatory process in Japan can be complex and often requires companies to repeat clinical trials for local populations, it recognizes the principle of individual variation and advantages products developed with the Japanese population in mind.

“The Japanese pharmaceuticals and Medical Devices Agency (PMDA) requires that a phase I study be conducted in Japanese patients, even if a

“These studies often reveal important genetic variation and patient response to medications among various populations.”

—Stephen Eck, M.D., Ph.D., Vice President, Oncology at Japanese drugmaker **Astellas Pharma** and PMC Board member

similar study was performed elsewhere,” noted Stephen Eck, M.D., Ph.D., Vice President, Oncology at Japanese drugmaker **Astellas Pharma** and PMC Board member, “These studies often reveal important genetic variation and patient response to medications among various populations.”

According to Mikio Kawahara, COO of the personalized medicine services and diagnostic organization **Riken Genesis**, “Compared to [government] actions supporting personalized medicine in the United States or Europe, Japan might be slightly behind. But people in government are working hard to advance personalized medicine...[to take] advantage of existing programs.”

Personalized Medicine Requires More Governmental Action

These six countries present six different scenarios for government-support of personalized medicine research and adoption. They all illustrate that while personalized medicine is complicated, more coherent governmental action can help build the institutions and infrastructure to support its development and the adoption of personalized medicine.

PMC Takes Stock

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Its themes, outlined in the above cover story and amplified in an op-ed authored by one of the conference participants, J. Leonard Lichtenfield, M.D., Chief Medical Officer of the American Cancer Society, suggest that policymakers must see the cancer ecosystem in its entirety if we are going to make progress in the future.

In her column, Amy Miller, PMC Vice President for Public Policy, discusses PMC’s analysis of on-going organizational and structural developments in comparative effectiveness research and our effort to encourage the leaders of that movement to remember we no longer live in a one-size-fits-all world.

And finally, to put personalized medicine in its proper international perspective, we analyze initiatives around the globe, noting that while no country has marshaled all

its resources to promote the paradigm, many are intrigued by the opportunity to improve clinical care and address the cost conundrum of health care.

In the spirit of the season, we ask that you help us to continue activities such as these in 2013 by joining us or renewing your membership next year.

We see three big and contentious challenges in the public arena: regulation, reimbursement, and education. As we know, the regulation of diagnostics, especially in the United States is divided among different agencies with different rules and different cultures. To bring some clarity to the field, PMC has commissioned a landscape analysis to better understand the space; and for the first time, define how personalized medicine products are regulated and brought to market. We hope this analysis

helps set the stage for developing future consensus around diagnostics regulation for co-developed products, the hallmark of personalized medicine.

In addition, in concert with patient advocacy groups and the insurance industry, PMC, partnering with **BIO**, will organize a summit to consider what kinds of evidence should determine coverage and payment decisions for personalized medicine and how we can best develop the standards and data requirements to make those decisions.

And finally, PMC will continue to lend its efforts to building a personalized medicine informed health care workforce by educating providers at the front lines of clinical care about the power of personalized medicine, a necessary if not sufficient ingredient to ensure the adoption of these new innovative products.

Who Will Lead Us As We Turn The Tide Against Cancer?

BY J. LEONARD LICHTENFELD, M.D., | DEPUTY CHIEF MEDICAL OFFICER, AMERICAN CANCER SOCIETY

We have developed incredible science and inconceivable opportunities to understand and treat cancer. But despite scientific progress, the cancer community is still challenged to bring about many changes to the health care system necessary to make our vision of personalized medicine a reality and a commercial success.

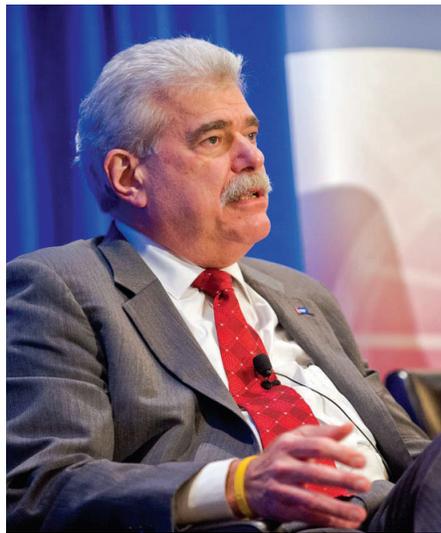
The progress we have made in the laboratory over the past 40 years has been truly incredible. We have learned much about what makes a cancer cell a cancer cell. We have made the first steps in harnessing that knowledge to bring new treatments to our patients that are crafted to interfere with the internal features of individual cancer cells. We are moving into an era where traditional microscopic examination of cancer tissues will be relatively outmoded, and genomic analysis of cancers will be a routine part of the decision-making process to guide treatment selection.

Cancer is a complex disease with many ways to circumvent our discoveries and our treatments. Although the science is promising, there remain many sobering realities that we must address.

Money is tight everywhere and research budgets are being cut, but nevertheless, we need to continue our research. It takes investigators a lifetime of work and discovery to lead to new paths for success. If we don't have the resources to fund young investigators—and maintain our established research workforce—we will pay a significant penalty in years ahead.

We must also fix the outmoded process that drives drug development. Pharmaceutical companies and venture capitalists are being asked to invest in the development of new drugs, engage in clinical trials, and bring new drugs to market. But the risks are mounting in these enterprises, and successes are becoming ever more expensive with a declining return on investment. As we

slice and dice the cancer genome, we find that there are many individual variations in the genetic makeup of our cancers. The drugs that are developed will be useful in smaller and smaller segments of the cancer patient population. There comes a point when investments in research and development become so high that they are not made because no patient will be able to afford to receive the drug and no payer will be willing to offer it.



J. Leonard Lichtenfeld, M.D., Deputy Chief Medical Officer, American Cancer Society

Biomarkers will hopefully help us to more precisely target patients who will benefit from new drugs. Patient targeting will reduce the costs of drug development by allowing for more productive clinical trials with smaller patient samples, but additional barriers to research and drug development still stand in the way of potential successes in cancer drug development.

Academic researchers and drug companies have traditionally been very proprietary when it comes to their research and their data. Keeping data in a silo, protected from the peering eyes of others, has been the rule. However, if we are going

to take advantage of the new world, we are going to have to share data and ideas.

New models of research collaboration among experts around the country and around the world are going to have to become the norm. Genomic data libraries have to be designed, funded and made readily available. How we structure clinical trials will have to change because finding patients with a specific cancer and a specific genomic abnormality in that particular cancer is going to be increasingly difficult. Instead of herding 50 patients with lung cancer into a major academic research center and trying out a new drug, we must instead search for patients with specific genetic changes in their cancers and involve them in clinical trials.

But to identify patients across the country (or around the world) by individual cancer mutation, we must also build and implement new information systems that are standardized, easily exchange information and protect patient privacy.

Then, there is the matter of costs.

Who is going to pay for all this? Not just the research and the researchers, but the clinical trials, the costs of the drugs and diagnostics, and the implementation of a robust health information system as well as the cost of caring for all cancer patients in our aging population.

Can we make this work? Can we get everyone in line and on board? Can we make certain that we build in the right quality and ethical protections for our patients? Can we pay for it?

All of those are daunting tasks. But ultimately as our scientific understanding of cancer biology grows enabling more precise molecular diagnoses and more targeted personalized medicines, we must also continue our commitment to ensuring that patients have access to the best treatments for their individual cancers—whether in the community clinic or in the world's best academic medical centers.

Personalized Medicine Coalition Announces New Board Appointments

The Personalized Medicine Coalition has elected four leaders in personalized medicine to its board of directors: Paul R. Billings, M.D., Ph.D., chief medical officer at **Life Technologies**; Donna R. Cryer, J.D., President and CEO of the **American Liver Foundation**; Timothy J. Garnett, M.D., senior vice president and chief medical officer for the Development Center of Excellence & Lilly Research Laboratories at **Eli Lilly and Company**; and Michael J. Vasconcelles, M.D., senior vice president, **Millennium: The Takeda Oncology Company**.

“The new board members represent the multiple voices within the personalized medicine community. Their diverse experience will enhance PMC’s ability to carry out its mission. They bring a wealth of knowledge needed to sustain personalized medicine innovation” said Nancy Simonian, M.D., chair of the nominating committee for the PMC Board of Directors.

Through his work at Life Technologies, Dr. Paul Billings, a Board certified internist and clinical geneticist, has sought to improve patient care by expanding the use of genomics technologies in clinical practice. Billings founded several genetics-focused companies and previously served as the director and chief scientific officer of the Genomic Medicine Institute at **El Camino Hospital**. A former member of the Secretary’s Advisory Committee on Genetics, Health and Society (SACGHS), he combines a knowledge of science, medicine and policy.

Donna Cryer is the president and CEO of the American Liver Foundation, the largest and oldest national non-profit organization serving liver disease patients and their families. Cryer was previously the chief executive officer of **CryerHealth**, an international health care consulting firm focused on patient engagement and alliance development. A tireless advocate for patients and personalized medicine, Cryer is also currently serving a five-year term as a patient representative to the U.S. Food and Drug Administration.

Timothy Garnett was part of the team that developed Eli Lilly and Company’s strategic approach to tailored therapeutics. As the co-leader of the company’s Development Center of Excellence, he is responsible for finding ways to implement personalized medicine in all aspects of drug discovery, development and commercialization. Prior to joining Lilly 14 years ago, Garnett worked for Organon Laboratories in Europe, and practiced obstetrics and gynecology.

With extensive experience in oncology drug development, Michael Vasconcelles brings first-hand knowledge of how companies can employ targeted approaches to improve patient selection for clinical trials and companion diagnostics in drug development. Previously, Vasconcelles was the Global Therapeutic Area head for transplant and oncology at Genzyme. He is also a clinical instructor of medicine at Harvard Medical School where he has been on the faculty since 1996.



New board members: Left, Paul R. Billings, M.D., Ph.D., Donna R. Cryer, J.D.; Timothy J. Garnett, M.D.; Michael J. Vasconcelles, M.D.

PMC IN THE PRESS

A special sixteen page supplement entitled, “Personalized Health: Up Close and Personal” was part of the September 28th issue of **USA Today**. PMC had the unique opportunity to provide editorial guidance to the supplement, which gave an in-depth and accessible account of personalized medicine to 1.4 million readers. PMC President Edward Abrahams contributed the foreword. The supplement also featured several PMC Board members, including Neil de Crescenzo, Bill Dalton, Larry Lesko, Clay Marsh and Jared Schwartz as well as many PMC members.

HealthLeaders Magazine discussed how many health care institutions across the country are beginning to adopt personalized medicine in cancer care and are demonstrating how the paradigm moves from bench to bedside, noted Edward Abrahams in an article about how cancer is treated at institutions such as the **Moffitt Cancer Center, Knight Cancer Institute and Mayo Clinic**.

PMC President Edward Abrahams and Susan Garfield of **GfK Bridgehead** authored a “Call to Action” essay for **European Pharmaceutical Contractor** magazine outlining the need for European governments to improve their regulatory systems in order to spur innovation and improve patient access to new personalized medicine diagnostic tests.

A new study as part of the Cancer Genome Atlas found that there are four main breast cancer subtypes, and a pair of articles in **Bloomberg** and **BusinessWeek** discussed the potential for targeted therapeutic approaches based on the molecular bases of the tumors. The articles cited data from the Personalized Medicine Coalition’s *Case for Personalized Medicine* regarding the growth in available personalized medicine products.

An article in **GenomeWeb** discussed the Patient Centered Outcomes Research Institute’s draft methodology report, and quoted PMC Vice President for Public Policy Amy Miller. “Right now I am not convinced that the report adequately addresses personalized medicine as an important factor in improving health outcomes through patient-centered outcomes research methodology,” she said. Miller encouraged PMC members to join the Coalition in calling on PCORI to make the final version more focused on personalized medicine. “Active input from the community is required so that the research will live up to the high expectations that we have for it,” she added.

In July, the American College of Cardiology’s **ACC in Touch Blog** discussed personalized cardiovascular medicine and the opportunity to reduce “unexpected drug-drug interactions” and by offering patients “better and more coordinated care.” The article cited PMC in defining the field, and highlighted data about physician knowledge of personalized medicine tests from ACC’s CardioServe panel survey which was used to inform ACC’s presentation at PMC’s 2011 conference *New Frontiers in Personalized Medicine: Cardiovascular Research and Clinical Care*.

When **Bristol-Myers Squibb Company** and **Eli Lilly and Company** won FDA approval in July to use their head and neck cancer medicine Erbitux for colorectal cancer and approved Venlo, a companion diagnostic test developed by **QIAGEN, Inc.**, **Bloomberg** covered the story, noting the growth in the use of diagnostics to guide treatment decisions. The report cited data from the Personalized Medicine Coalition about the growing personalized medicine trend.

Harvard biomedical informatics expert Mark Boguski launched a new company, Genome Health Solutions, aimed at helping to bring genomic analysis into wider use. In a July interview with **Genetic Engineering & Biotechnology News**, PMC President Edward Abrahams noted, “[Boguski is] a leading pathologist [who] recognizes that there are intervening steps between the technology and improved outcomes.”

Key themes from *Turning the Tide Against Cancer For Sustained Medical Innovation*,

PMC’s conference in collaboration with the American Association for Cancer Research and Feinstein Kean Healthcare were highlighted in **The ASCO Post**. The conference, held June 12, 2012 in Washington, DC was also highlighted on numerous blogs including **The Age of Personalized Medicine**, PhRMA’s Blog of **The Catalyst**, **Dr. Len’s Cancer Blog**, the **Stand Up 2 Cancer Blog**, and the National Brain Tumor Society website.

Amy Miller was also quoted in a **Nature Biotechnology** story about comparative effectiveness research and the role of PCORI as the new gatekeeper to this health care concept thought to help improve health outcomes while lowering overall costs. “We think PCORI was designed to answer specific questions about the health care system that Americans need answered,” she said, “I don’t think that by designing these research priorities to be as vague as they are, that PCORI will get to that.”

