

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

Biopharmaceutical Industry Remains Firmly Committed to Personalized Medicine Despite Challenges

“Personalized medicine is good for patients, it’s good for health care, and it’s good for the economy,” John J. Castellani, President and CEO of the **Pharmaceutical Research and Manufacturers of America (PhRMA)**, told an audience of nearly 200 officials from industry, government, and academe at the Personalized Medicine Coalition (PMC)’s Seventh Annual State of Personalized Medicine Luncheon at the National Press Club on June 25.

In the first speech focused on personalized medicine since he took the helm at PhRMA last year, Mr. Castellani emphasized the biopharmaceutical

sector’s commitment to personalized medicine despite the complexities of applying insights from recent discoveries in genetics and molecular biology to research and development. Personalized medicine has the potential to improve health outcomes and bend the cost curve by making our health system more efficient, he said. He also discussed policy changes needed to accelerate the development and adoption of personalized approaches to care, which would create high quality jobs to aid the economic recovery.

Citing a recent survey by the *continued on page 4*



ELLIE VAN HOUTTIE PHOTOGRAPHY

John J. Castellani speaking at PMC’s Seventh Annual State of Personalized Medicine Luncheon.

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FROM THE PRESIDENT



Global Opportunities, Challenges Face Personalized Medicine

BY EDWARD ABRAHAMS

The Personalized Medicine Coalition is an educational and advocacy organization whose aim—to create a friendlier environment for personalized medicine—is, like its membership, global in its orientation: of our 220 members, nearly 20 percent have their headquarters outside the United States.

I just returned from a trip to Switzerland and Germany. In Geneva, I co-chaired a conference on theranostics.

In Cologne, I was the keynote speaker at a conference sponsored in part by North Rhine-Westphalia and **QIAGEN**, the largest independent diagnostic company in the world and a PMC member, focusing attention on opportunities and challenges in personalized medicine.

In May, I participated in a conference in Brussels, organized by the European Commission, entitled, “European

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POLICY UPDATE

Workgroup Crafts Legislative Recommendations

The PMC legislative workgroup finalized legislative recommendations for congressional consideration in advance of the introduction of a new version of the Genomics and Personalized Medicine Act. Last year's bill contained some outdated provisions and others that failed to facilitate the development of personalized medicine. The recommendations focus on clarifying the regulatory process, improving reimbursement policy and creating business incentives to spur innovation.

FDA Releases Draft Guidance on IVD Products

The Food and Drug Administration (FDA) released draft guidance entitled "Commercially Distributed In Vitro Diagnostic Products Labeled for Research Use Only or Investigational Use Only: Frequently Asked Questions." The guidance will affect "research-use only" and "investigative use only" diagnostics, which do not require FDA premarket review before being distributed for investigative purposes. FDA notes that some research diagnostics have been marketed for clinical use, even though FDA has not approved them and they may cause serious adverse results for patients. The guidance is meant to remind manufacturers of the rules for research and investigative diagnostics.

The draft guidance is available in the federal register for June 1 at <http://www.gpoaccess.gov/fr/>. Comments on the draft guidance may be submitted in writing or electronically at <http://www.regulations.gov> and are due by August 30.

BIO Convention Panel Discusses Diagnostic Reimbursement Policy

PMC Public Policy Director Amy Miller hosted a panel discussion of reimbursement issues facing advanced diagnostics at the **BIO** International Convention on June 29. Panelists including Scott Allocco of **Biomarker Strategies**, David Parker of **Boston Healthcare**, Steve Phillips of **Johnson & Johnson** and Kristen Porthier of **Health Advances** discussed key themes from the reimbursement paper PMC released last fall and a separate paper by Health Advances released earlier this year. At the conference, PMC and BIO announced that they will continue this work.

Cardiovascular Conference Summary Available Online

The National Heart Lung and Blood Institute (NHLBI) has posted a summary of conference proceedings as well as a list of recommendations generated from *New Frontiers in Personalized Medicine: Cardiovascular Research and Clinical Care* conference on its website at <http://www.nhlbi.nih.gov/meetings/workshops/joint-cardiovascular.htm>. The conference, held at **The George Washington University** (GWU) on January 6 and produced by PMC in collaboration with NHLBI, GWU, the **American Medical Association** and the American College of Cardiology, brought together leaders from academia, industry, and government to discuss the science, business, and policy of cardiovascular applications of personalized medicine.

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Sausage Making at Its Finest

BY AMY MILLER | PUBLIC POLICY DIRECTOR

This spring we organized a Capitol Hill briefing (see page 5) to inform Congress about the challenges facing personalized medicine in advance of the reintroduction of the Genomics and Personalized Medicine Act (GPMA). After that briefing, we met with several members of Congress and key staff to discuss personalized medicine and to gauge interest in and commitment to personalized medicine. Given the challenges facing legislators as they negotiate a compromise to raise the debt ceiling, it's clear that deficit reduction is guiding every decision Congress makes. Thus, it was not surprising that legislators responded favorably to our contention that personalized medicine will bring cost savings to the health care system by eliminating the waste that is inherent in trial-and-error medicine, and wanted to know what they could do to help advance the paradigm.

But even those legislators who have a positive view of personalized medicine don't necessarily know what policies can best move it forward. At our spring public policy committee meeting, guest speaker Erin Katzelnick-Wise, legislative aide to Rep. Anna Eshoo (D-Calif.), the champion of GPMA in the House of Representatives, told us that Rep. Eshoo wants to introduce a new version of the bill that would enjoy wide community support and asked for our assistance in putting together that

legislation. Since then, PMC's legislative workgroup has developed specifications to further articulate the organization's defining principles and to highlight new policies that would further facilitate

the development of personalized medicine.

PMC's proposed package of legislative specifications would incentivize personalized medicine by:

- creating a stable and predictable regulatory

environment for personalized medicine products under review at FDA;

- providing research and development tax credits for companies developing personalized medicine drugs and diagnostics;
- ensuring adequate representation of personalized medicine experts on relevant federal committees;
- helping ensure patient access to personalized medicine through Medicare coverage of personalized medicine diagnostics;
- developing an expedited exceptions process for personalized medicine therapeutics; and
- requiring the Center for Medicare and Medicaid Innovation to test programs that would promote personalized approaches to health care.

Legislators who have a positive view of personalized medicine don't necessarily know what policies can best move it forward.

While analyzing the policy barriers to personalized medicine, the workgroup noted that some barriers could be tackled through direct advocacy rather than legislation. For example, as outlined in our white paper on reimbursement barriers to personalized medicine published last fall, the current system for coding, coverage, and reimbursement of diagnostics disadvantages personalized medicine. To address this challenge, we are working with the **American Medical Association** (AMA) and encourage interested PMC members to participate in AMA's Current Procedural Terminology (CPT®) Editorial Panel Molecular Pathology Coding Workgroup activities. We look forward to working directly with

AMA to help it modernize the CPT coding system in order to ensure that patients have timely access to the latest innovations in personalized medicine.

PMC's workgroup will also examine how to best structure market incentives and develop a reimbursement framework for genetic counseling services. PMC has long advocated for a patent exclusivity extension to help incent developers to pursue personalized medicine product development. Since this is

a hard sell politically in the current fiscal environment, we will carefully craft the arguments for these incentives before we bring them to Congress.



Rep. Anna Eshoo (D-Calif.) plans to introduce a new version of the GPMA later this year.

Solutions Needed to Accelerate Adoption of Personalized Medicine

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Tufts Center for the Study of Drug Development as evidence of the sector's commitment to personalized medicine, he said, "Ninety-four percent of biopharmaceutical companies are investing in personalized medicine research, and 100 percent said they are using biomarkers in the discovery stage to learn more about compounds."

With these figures, combined with the statistic that the biopharmaceutical industry has increased its investment in personalized medicine by 75 percent, Castellani countered oft-repeated criticisms of his industry's lack of commitment to personalized medicine.

"The era of one-size-fits-all medicine is over," said Edward Abrahams, discussing the impact of Mr. Castellani's remarks. "The news coming out of the American Society of Clinical Oncology Annual

Mr. Castellani also addressed recent criticisms that the human genome project has not sufficiently transformed patient care since its completion a decade ago.

"While we are humbled at the complexity of the science [of human disease] and the challenges of translating it into better tests and treatments, this should not be a reason for us to reduce our effort, but rather to redouble it. Patients like Adriana demand that we do," he noted, referencing the dying wish of Adriana Jenkins, a breast cancer patient whose plea to the pharmaceutical industry to create more drugs like Herceptin®, which she credited with extending her life by nine years, was

importance of partnerships to overcome the many scientific, regulatory, and policy barriers to adoption and implementation of personalized

medicine—a theme echoed throughout the keynote. "To bring personalized medicine forward, scientists, regulators, policymakers, physicians, the patient community, payers, and pharmaceutical

and diagnostics companies must work in concert," said Mr. Verbiest. "We must find ways to more successfully work together, adopt a new mindset, and pursue a more open, 'networked' approach to innovation."

Mr. Castellani discussed the need for solutions to enable the acceleration and adoption of personalized medicine, including changes to Food and Drug Administration regulations, reimbursement models, comparative effectiveness research and health information technology.

"Federal regulation of diagnostic and laboratory testing remains unclear, creating uncertainty for innovators and providers. Efficient pathways for premarket review of drug/diagnostic combinations need to be more clearly defined by FDA," he said.

Meanwhile, as Washington focuses on deficit reduction and job creation, he underscored the value of personalized medicine in advancing both goals by discussing the potential savings to the health care system of preventing adverse events and avoiding unnecessary or ineffective treatments. He cited warfarin sensitivity testing and determining whether or not women with breast cancer would benefit from chemotherapy after surgery as examples.

"Our latest data shows that the biopharmaceutical sector directly supports more than 650,000 jobs and indirectly fuels more than 2.4 million additional jobs in other sectors such as suppliers and service providers to the industry," noted Mr. Castellani.

Ninety-four percent of biopharmaceutical companies are investing in personalized medicine research.



ELLIE VAN HOUTTE PHOTOGRAPHY

John J. Castellani fields questions offered by PMC President Edward Abrahams at PMC's annual luncheon.

Meeting on the successful clinical trials of targeted therapies including crizotinib and vemurafenib demonstrates that the biopharmaceutical industry has embraced the principle of pinpointing disease at the molecular level to ensure that the right patients receive the right treatments."

published in *Forbes* shortly after her death in February (and republished in PMC's Spring newsletter).

Werner Verbiest, head of **Johnson & Johnson's** Companion Diagnostics Center of Excellence, introduced Mr. Castellani and highlighted the

Congressional Briefing Underscores Need for Public Policies Supporting Personalized Medicine's Continued Development

Personalized medicine is very early in its life cycle, but it is rapidly improving health care quality for patients, panelists at a PMC-hosted congressional briefing told a packed room of more than 70 congressional staff and industry leaders.

In collaboration with House Medical Technology Caucus co-chairs Rep. Erik Paulson (R-Minn.) and Rep. Anna Eshoo (D-Calif.), PMC's briefing, "Personalized Medicine 101: How Medical Technology Can Improve Health" was designed to inform members of Congress and congressional staff about personalized medicine and to demonstrate how the tailoring of medical treatments to the individual characteristics of each patient can make health care better and more efficient.

"Health care reform raised the profile of personalized medicine on Capitol Hill but now that deficit reduction dominates the conversation in Washington, many members of Congress and congressional staffers want to better understand how personalized medicine can improve health outcomes while lowering health care costs," said Amy Miller, Ph.D., Public Policy Director for the Personalized Medicine Coalition.

"The challenges facing personalized medicine are not limited to gaps in science; significant public policy hurdles must be addressed before patients have adequate access to tailored therapies," said Brian Munroe, founder of the PMC and head of **Endo Pharmaceuticals'** Washington office.

The science of new drug development has, in many ways, already made the transition from "one-size-fits-all"

treatments; many innovators recognize that they must work with subpopulations and use genetic and biomarker data to inform drug discovery.

Panelist Stephen Eck, M.D., Ph.D., Vice President and Global Head of Medical Oncology for **Astellas Pharma Global Development**, discussed the science of personalized medicine and

system, especially in health information technology, to integrate data, store information, and ultimately guide physicians as they match treatment to patient at the point of care.

"Personalized medicine is not a vision for the future; when I see patients, they want and must get treatments that will work for them today," explained Dr. Abernethy.

Offering the patient perspective, panelist Donna Cryer, CEO of **CryerHealth** and Chair of the American Liver Foundation's Board of Directors, addressed firsthand the benefits offered by a new generation of personalized medicine diagnostics. A liver transplant recipient, she discussed how regular immunologic testing helps her avoid costly hospitalizations due to infection or transplant rejection.

"Personalized medicine is the ultimate patient-centric solution, giving patients greater confidence

in their diagnoses and treatment selection," noted Ms. Cryer, "but low reimbursements threaten patient access to innovative diagnostic tests. Without higher reimbursements, diagnostics companies will not be able to recoup their R&D investments and tests that improve patient quality of life while lowering health care costs will not come to market."

PMC developed a package of incentives for congressional consideration that will advance personalized medicine to ensure that innovation continues, that physicians have the tools they need to offer patient-centered care, and that patients have access to the best medicine science can offer.



ELLIE VAN HOUTTE PHOTOGRAPHY

Panelists Stephen Eck, M.D., Ph.D., Donna Cryer, and Amy Abernethy, M.D. discuss the science behind personalized medicine and some of the legislative and administrative challenges the health care industry faces.

the biopharmaceutical industry's commitment to improving health outcomes.

"A recent study released by the Tufts Center for Drug Development notes that personalized medicine products comprise between 12 and 50 percent of current drug pipelines," he said. "But challenges remain in educating patients and physicians about the genetic mechanisms of disease and the benefits of personalized medicine."

Speaking about her work as a practicing oncologist, panelist Amy Abernethy, M.D., Director of the **Duke Cancer Care Research Program** at Duke Cancer Institute, highlighted the need for changes across the health

From Genealogy to Personalized Medicine: Utah Attracts Top Scientists

Family genealogy records dating back to the 1800s kept by the Church of Jesus Christ of Latter-Day Saints (the Mormon church) were key to Utah's emergence as a leader in personalized medicine. When geneticists asked church members in the 1970s to share the information and also provide DNA samples, they agreed, initiating a boom in genetic research and the launch of several personalized medicine-focused companies.

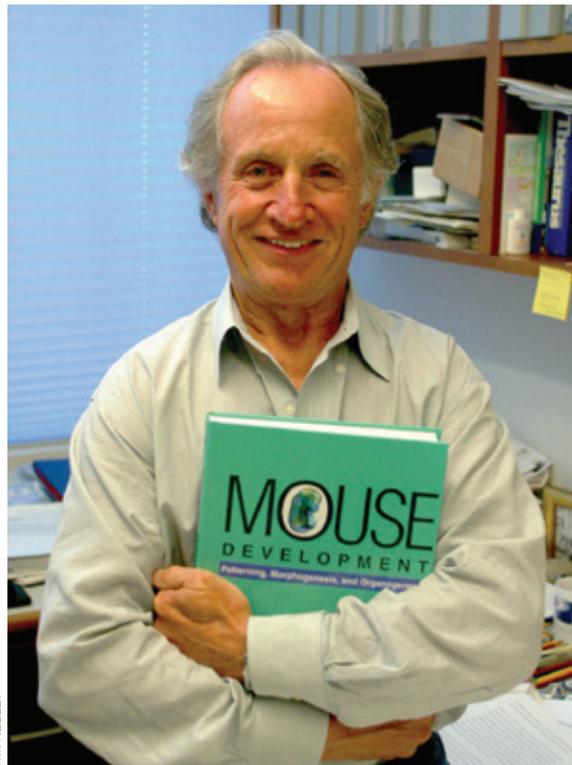
Bradley Cairns, Ph.D., senior director of basic science at the Huntsman Cancer Institute in Salt Lake City, which aims to develop individualized treatments based on the genetic makeup of both patients and tumors, says this deep pool of data from large families with several generations still living, combined with the number of scientists from many fields who are seeking to use it for new discoveries, makes Utah a unique place for scientific research. "We have families with a predisposition to cancers or diseases. We have teams of epidemiologists to research genetic mutations," he said.

University of Utah serves as catalyst for innovation

"We are about more than incorporating genetic research. We are personalizing our health care system to enhance individual patient care by personalizing their susceptibility, diagnoses, prognosis and treatment. We are facilitating not only patient care but research and education as well," said Michael Varner, M.D., interim director of the **University of Utah** Program in Personalized Health Care.

Utah's scientific contributions in genetics and biology were recognized with a Nobel Prize in 2007. Mario R.

Capecchi, Ph.D., distinguished professor of human genetics and biology at the University's Eccles Institute of Human Genetics, won the prize for pioneering development of "knockout mice" technology, a gene-targeting technique



TIM KELLEY

Mario R. Capecchi, Ph.D., a professor at the University of Utah's Eccles Institute of Human Genetics, won the Nobel Prize for pioneering development of a gene-targeting technique in mice.

that allowed the creation of animal models for hundreds of human diseases, including cancers in mice.

On the education front, the university is helping to raise a generation that understands the science behind personalized medicine through professional development forums for educators. It is also bringing gene science to the masses through its teaching website, Learn.Genetics™ (<http://learn.genetics.utah.edu>), one of the most

widely-disseminated education websites in the world, which has received in excess of 7.1 million unique visits from more than 180 countries annually. It also partnered with The Leonardo Museum to build an interactive genomics exhibit, opening in September, where visitors will be able to submit DNA samples for interdisciplinary research into genes' connections with cognition and behavior.

Scientific discoveries spawn personalized medicine-focused businesses

In 1980, Mark Skolnick, Ph.D., and Raymond White, Ph.D., both of whom were at the University of Utah, were two of the four researchers who proposed a method for constructing a genetic linkage map using restriction fragment length polymorphisms (RFLP) that was used in subsequent years to identify several human disease genes including Huntington's disease and breast cancer predictor gene BRCA1. Dr. White went on to found the Huntsman Cancer Institute. In 1991, Dr. Skolnick co-founded Utah-based Myriad Genetics, which among other diagnostic tests offers one for breast cancer predisposition.

Bill Harten, a computer scientist who developed GEDCOM, an open-source program that enables different programs to share genealogy information for the Mormon church, worked with Myriad Genetics' scientists to develop the sophisticated software they required to process the enormous amount of data involved in their genetic and genomic research. He also worked with **Celera** on the Human Proteome Project, among others. From that origin, a technology and company evolved, **UNICconnect**,

a company which provides software platforms that manage gene data workflow.

One of UNICConnect's clients is Utah-based **Axial Biotech**, a molecular diagnostics company that developed the ScolioScore™ test. The test, launched in 2009, helps determine whether or not scoliosis requires aggressive treatment by using a panel of genes to predict the risk of disease progression. Other clients include Clariant, which was recently purchased by **GE Healthcare**, and Vivia Biotech, based in Madrid.

Another Utah company, **Lineagen, Inc.**, introduced the FirstStepDx Test this year. The test is designed to help physicians evaluate and treat children with autism spectrum disorder and developmental delays.

Political and thought leaders support personalized health care

The state's political leaders have also played a key role in Utah's emergence as a leader in personalized medicine. Michael O. Leavitt, who served as both the state's governor and as secretary of the U.S. Department of Health and Human Services, used his authority to bring resources and attention to personalized medicine both in the state and in Washington, D.C. Republican Sen. Orrin Hatch of Utah has also played a key role in promoting personalized medicine on Capitol Hill.

Since 2008, leaders in personalized medicine from Utah and around the country have hosted the



UNIVERSITY OF UTAH

The Huntsman Cancer Institute in Salt Lake City is a National Cancer Institute-Designated Cancer Center, which means it meets the highest national standards for cancer care and research and receives government support for its scientific endeavors.

Personalized Health Care Summit in Deer Valley, Utah. At the 2010 forum, Utah native Clayton Christensen, DBA, Harvard University Business School Professor and author of *The Innovator's Prescription*, kicked off an effort to develop a "Roadmap for Personalized Health Care" to highlight the ways in which personalized medicine will upend traditional medicine.



Clayton M. Christensen, DBA, has described personalized medicine as a disruptive innovation.

Dr. Christensen said he attributes much of the energy in Utah to the Huntsman Cancer Institute. "Cancer is one area where we are now identifying the disease by cause rather than symptom, so personalized medicine is really

important," he said. "There was a time in cancer that the paradigm was geographic: breast, brain, bone, blood. Now, he noted, "we've got several thousand types of cancer identified," but new research must be integrated into clinical practice in order for the paradigm to be put in place.

Dr. Christensen's Roadmap for Personalized Health Care will lay the groundwork for this necessary transition. Slated for release later this year, it will be based on Christensen's analysis of how disruptive innovation can be harnessed to bring about top-to-bottom changes in the health care system.

"This is not like Route 66," he said. "In order to make any progress at all, you have to get through intersections—and you can't get through them unless all of the other players arrive at the intersection at the same time." The road map will show those "intersections" or nodes where different stakeholders will need to be in synch on solutions, he said.

Prizes Speed Technological Innovation

Several institutions are trying to speed up the use of cutting-edge personalized medicine technologies with a centuries-old approach for overcoming major scientific hurdles: cash prizes.

The biggest prize is the \$10 million **Archon Genomics X PRIZE** administered by the X PRIZE Foundation, which will go to the first team that can build a device and use it to sequence 100 human genomes within ten days. Dr. Stewart Blusson, a former scientist with the Canadian Geological Survey who discovered diamond mines in Canada, and his wife Marilyn are lead sponsors of the prize. Other supporters include the J. Craig Venter Institute and **FasterCures**, among others.

Similarly, **Life Technologies** is offering \$1 million prizes to individuals or teams that can meet more narrowly-defined challenges that would improve its sequencing devices.

Both prizes aim to reduce the cost and improve the efficiency of sequencing the genome, a building block of personalized medicine. Commercially, the cost of sequencing the genome now stands at \$5,000, far less than it was five years ago. As it drops further, sequencing patients' DNA will become a diagnostic tool and gene data will become part of a patient's medical record, thus enabling the matching of therapy to patient based on patient genetics.

Competitions such as these are often described as "crowdsourcing," a way of recruiting a large number of outsiders to solve problems, which today is often associated with use of the Internet to leverage and aggregate knowledge. However, the competition approach to advancing technology dates back at least to 1714, when the British government offered a prize to whoever came up with a way to determine a ship's longitude while at sea.

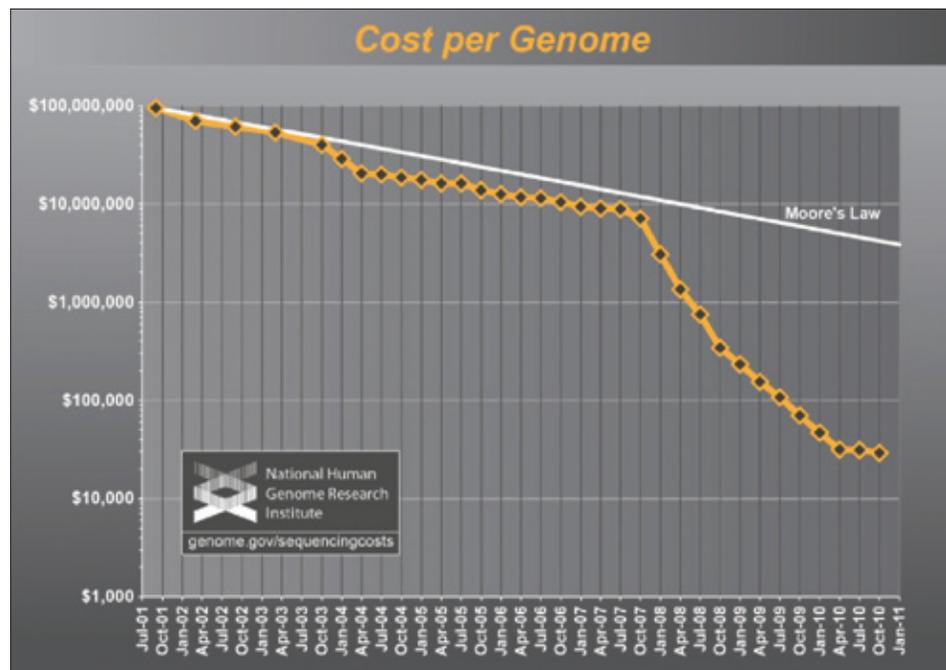
More than £100,000 (about \$160,000) was distributed over the next five decades to eight people who developed or refined the marine chronometer.

The X PRIZE Foundation is hoping its genome-sequencing challenge can be met a little more quickly to speed the use of sequencing technology and advance medical care by making sequencing accurate, yet less expensive.

"Dramatic improvements in medical treatments will almost certainly occur

cost. They must have no more than one error in every 100,000 bases sequenced, with sequences accurately covering at least 98 percent of the genome, at a cost of no more than \$10,000 per genome.

So far, a total of eight teams have registered for the Archon Genomics X PRIZE since it was announced in 2006,



The falling cost of sequencing the genome: It took \$3 billion and 13 years from 1990 to 2003 to read out the first draft of the human genome. From 2005 to 2008, however, second generation DNA sequencing instruments were introduced and broadly taken up in the research market. By 2009, the cost and duration of sequencing an entire genome decreased significantly to \$50,000 and two months.

when individual patients get their complete genomes sequenced and their medical decisions are based on this information," said Edward Rubin, M.D., Ph.D., Director, U.S. Department of Energy's Joint Genome Institute. "While almost in our grasp, its clinical reality awaits radically new technologies and massive reductions in DNA sequencing cost, features that the Archon Genomics X PRIZE will both encourage and accelerate."

To win the X PRIZE, a team must sequence 100 genomes in ten days, and must meet strict criteria for accuracy and

including Harvard genetics professor and Human Genome Project originator George Church.

Life Technologies believes its prizes are the first crowdsourcing campaign focused on a biological and medical laboratory tool. "We want to speed innovation and development on our platforms," said Paul Billings, M.D., Ph.D., Chief Medical Officer of Life Technologies. "Obviously, we've put hundreds of millions of dollars into our own research, but we're hoping to stimulate ideas through the sharing of knowledge, expertise and innovation."

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Personalized Medicine: The Payer Perspective

BY DAVID G. BROOKS, M.D., PH.D., CHIEF MEDICAL OFFICER, GENERATION HEALTH, AND TROYEN A. BRENNAN, M.D., EXECUTIVE VICE PRESIDENT AND CHIEF MEDICAL OFFICER, CVS CAREMARK

The era of personalized medicine is upon us. Nowhere is this more evident than in oncology, where molecular classification of tumors by a diagnostic test result increasingly guides prescribing. Indeed, the revolutionary companion diagnostic paradigm is the underpinning of modern oncology, and personalized medicine (PM) solutions will no doubt soon proliferate in other disease categories. This is good news for patients.

However, PM is not a simple story of more information and better treatment. Biomedical research has spawned a plethora of candidate biomarkers that are touted as opportunities to personalize medicine, but generation and dissemination of knowledge about the usefulness of these tests has been relatively slow. Compounding the problem are the skyrocketing variety and sophistication of methodologies to interrogate these biomarkers/molecular tests. Keeping up with the pace and breadth of developments in this exciting field is beyond the scope of most physicians, and certainly beyond the scope of payers and managers of care.

The lack of internal expertise to manage this explosive growth in molecular testing is far from the only concern that payers have about PM. Other important issues include how well molecular tests work and are translated into actual clinical practice. Insurers worry that new, esoteric tests may not affect patient management and will add to, not replace, costs. Despite the fact that lab tests only represent 3-5 percent of expenditures today, payers are concerned with the 25-40 percent annual growth rate in spending on them. Payers often lack convincing evidence that the promise of PM leads to better outcomes at equal or lower costs.

Moreover, the PM field is moving fast. To this point, clinical utility and/or comparative effectiveness evidence typically have not been available when payers initially draft reimbursement policy, or have not been definitive enough to determine which interventions should be actively promoted. Historically, diagnostic tests have not been sufficiently reimbursed for their developers to mount the large and long-term studies required to generate strong evidence of improved clinical outcomes in a cost-efficient manner. In contrast with tight regulation on introduction of new prescription drugs, FDA does not regulate all diagnostic tests. Payers thus confront a landscape of laboratory developed tests where more than 5,000 labs generate multiple competing tests of variable quality, cost, specifications and reporting that are not FDA-regulated.

The consumer perspective must also be considered. A sick person naturally wants the best available information about

diagnosis and treatment options; however, the eagerness of some consumers to understand their personal and familial risks is easily exploited by companies. For example, a significant uptick in testing volume following direct-to-consumer marketing by companies involved in hereditary breast cancer screening has been observed, leading to testing of women who do not meet clinical criteria according to professional guidelines and health plan medical policies.

All of these issues are addressable. Government entities, payers and novel consortia of stakeholders are pushing personalized medicine into practice and trying to address the gaps in clinical utility and comparative effectiveness evidence; this effort is exemplified by the PCORI and Genetic Test Registry initiatives.

Private payers and pharmacy benefit managers are leading in one area of PM that is ready for translation to medical practice: pharmacogenomics (PGx). For example, **Caremark**, in collaboration with **Generation Health**, and **Medco** are each putting PGx programs into practice and ascertaining health economic and outcomes research (HEOR) endpoints of candidate PGx interventions. For example, Caremark and academic collaborators are tracking a clopidogrel PGx program to provide prospective evidence that avoiding clopidogrel in those predicted to have inefficient CYP2C19 metabolism leads to better outcomes. Large payers have shown similar initiative by researching their membership to track performance and generate evidence of the value of PM interventions, as illustrated by the recent collaboration of Wellpoint, HealthCore and AstraZeneca.

Organizations such as PMC also play an important role in convening scientists, payers, policy makers, legislators and regulators for the broad discussion necessary to advance the field. Collaboration is critical to allow for the development of utility evidence supporting PM interventions. In our view, this evidence will include timely comparative effectiveness research and rapid dissemination of data demonstrating improved outcomes.

Today we are witnessing the early promise of PM. Through the work of stakeholders and collaborators, we must help the field mature by providing the high quality, cost-effective health care patients deserve. Innovative collaborations and robust, real-world evidence will propel the achievement of the right medicine for the right patient at the right time.

The views expressed in this article are those of the authors and do not necessarily represent the opinions of the Personalized Medicine Coalition.

Payers often lack convincing evidence that the promise of personalized medicine leads to better outcomes at equal or lower costs.

Global Opportunities, Challenges Face Personalized Medicine

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Perspectives in Personalized Medicine,” which reviewed developments in the field to identify “future actions needed at the European level” to stimulate the advancement of personalized medicine, very much along the lines PMC advocates.

My chief takeaway from these conferences is that, with two important exceptions, the view from Washington regarding personalized medicine is not so different from those in Brussels, Geneva, or Cologne.

We face the same challenges, the same opportunities, and indeed, the same imperatives.

First, patients want and expect better health care. Given advances in science and technology, notably our ability to target treatments based on molecular and other diagnostic tools, their expectations are not misplaced. Second, health care systems across the world, no matter how

they are configured, are overburdened and stretched close to the breaking point. If we do not find new ways of delivering health care it will have to be rationed, either by the market or by governments. And third, the case for personalized medicine may be compelling and obvious to patients, but regulations, reimbursement systems, and educational infrastructures in Europe, as in the United States, inhibit investment in and adoption of the new paradigm.

However, the U.S. differs from Europe in two key ways. While the U.S. has a central government, that government, unlike European ones, has had little experience directing a coherent strategy in health care, the proposed experiment in health care reform enacted last year notwithstanding. On the other hand, while governance in Europe is less centralized, European governments have experience with single-payer systems that could help catalyze the development of

personalized medicine, if policymakers were so inclined.

Resources permitting, PMC plans to increase its activities in Europe. This fall, for example, at a **Cambridge Healthtech Institute** conference in Hanover, Germany, we will release a white paper on reimbursement policies across the continent to illustrate how personalized medicine products are paid for in Europe, along the lines of one we commissioned last year from **Boston Healthcare** on the U.S. reimbursement system.

Working with **Bridgehead International**, PMC is studying the experiences and attitudes of stakeholders in the European Union to understand the reimbursement landscape for personalized medicine diagnostics and therapies, including coverage, coding, payment and evidence standards. The white paper should be an invaluable product not only for our members but also for policymakers in Europe.

PERSONALIZED MEDICINE: 7th Annual Conference

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

The seventh annual Personalized Medicine conference will take place November 9-10, 2011 at The Joseph B. Martin Conference Center at Harvard Medical School in Boston. This year's two-day conference will highlight the current clinical impact of personalized medicine as it enters the healthcare delivery system and how recent experience may guide and inform the policies, plans and actions of stakeholders among government, academe and the private sector. Widely considered the premier event in the field, the conference attracts over 600 national and international thought leaders.



Clayton Christensen
Harvard Business School



Deborah Dursine
Millennium/Takeda



Michael Leavitt
Health & Human Services



Kathleen Behrns
KEW Group



Lord Naren Patel
House of Lords, UK

Personalized Medicine Coalition Announces New Board Appointment

The Personalized Medicine Coalition has elected Amy P. Abernethy, M.D., Director of the **Duke** Cancer Care Research Program, to its board.

Dr. Abernethy, an active clinician in both outpatient and inpatient oncology, is Associate Professor of Medicine at the Duke University School of Medicine, a Senior Fellow with the Duke Center for Clinical Health Policy Research, and faculty in the Duke Clinical Research Institute. She was recently appointed to the National Cancer Policy Forum with the Institute of Medicine.

“As a practicing physician who actively seeks personalized solutions for her patients, Dr. Abernethy brings a valuable perspective on the translation of genomic research into practice,” said Wayne Rosenkrans, Chairman of PMC’s board. Her work in comparative effectiveness research and patient-reported outcomes in cancer will broaden and deepen our understanding of how personalized and participatory medicine can demonstrate its value and be integrated into the health care system.”

“I am delighted and honored to join the Board of the Personalized Medicine Coalition,” Dr. Abernethy said. “As a cancer physician who thinks about how we are going to implement personalized medicine on the frontline in the clinic, I look forward to contributing to the Board as an advocate for personalized patient care now.”



Amy Abernethy, M.D.

Decision support systems are crucial to the effort, she said.

“We must integrate all available information whenever it is ready in order to best match interventions to improve care for individual patients. Efficient and effective clinical decision support systems are critical. As more data become available, especially biological information such as biomarker-based tests that inform the matching of cancer to cancer treatment, this information must be sequentially incorporated into the clinical decision support systems and everyday care,” she said. “Hence, our information systems

must be flexible enough to allow this sequential introduction of information as it becomes available so that we can stay most current as we personalize care. Personalized medicine is about taking care of people now and in our future.”

Dr. Abernethy, an investigator funded by the National Institutes of Health and the Agency for Healthcare Research and Quality, has a substantial portfolio focused on comparative effectiveness research and patient reported outcomes in cancer.

Dr. Abernethy founded and directs the Duke Cancer Care Research Program (DCCRP), which conducts a diverse portfolio of studies focused on improving the symptoms and quality of life of cancer patients at all stages of disease and survivorship.

Prizes Speed Technological Innovation

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Life Technologies is offering three \$1 million prizes to participants who can improve its bench-top genetic sequencing device, the Ion Torrent Personal Genome Machine, in specific ways: increase its output at least twofold; cut by half the time it takes to prepare a testing sample; and create an algorithm that at least doubles the accuracy of the machine’s DNA reads. A fourth prize will go to the first person to sequence the genome and transcriptome of a single cancer cell using the company’s SOLiD™ sequencer.

Meanwhile, the U.S. government is also using prizes in a way that will improve the health information technology infrastructure vital to the long-term

development of personalized medicine. A set of prizes announced in June by the Office of the National Coordinator for Health Information Technology are an effort to accelerate innovation and adoption of health information technology to improve clinical outcomes and make care delivery more efficient. The Investing in Innovations Initiative, or i2 prizes, will spend \$4.9 million over the next five years for information technology innovations designed to enhance data-sharing capacity among hospitals, clinics and physicians.

As noted in a report issued by the Brookings Institution in January, this interoperability is critical to the ongoing development of a health information

technology infrastructure that aggregates genomic data and facilitates the development of personalized approaches to care by advancing understanding of what treatments work for which patients through data aggregation, information-sharing, and rapid-learning feedback models.

Additional information on the prizes can be found at the following websites: Archon X-Prize: <http://genomics.xprize.org/>; Life Technologies: <http://www.lifetechnologies.com/content/lifetech/en/home/about-life-technologies/grand-challenges.html>; and i2 prizes: http://healthit.hhs.gov/portal/server.pt/community/healthit_hhs_gov__onc/1200.

PMC IN THE PRESS

Discussing a report by Scott Gottlieb, a fellow at the American Enterprise Institute, which said that comparative effectiveness research (CER) should not be a mandatory component of the drug approval process, PMC Public Policy Director Amy Miller was quoted in **FDA News**. “We’d hate to see a situation where a therapy is kept off the market because a CER study showed that the red pill treats a given condition better than the blue pill across the population,” she said.

BNA Life Sciences Law & Industry Report, FDA Week, Pharmacogenomics Reporter, and **The Pink Sheet** all covered the Seventh Annual State of Personalized Medicine Luncheon and keynote speech by John J. Castellani, President and CEO of **PhRMA**. The coverage focused on Mr. Castellani’s discussion of the biopharmaceutical industry’s commitment to personalized medicine, needed changes at FDA, and how to best use CER to guide health policy decision-making now that the Patient-Centered Outcomes Research Institute (PCORI) is up and running.

Reporting from PMC’s congressional briefing in May, **Inside Health Policy** noted that diabetes and mental health drugs represent the next frontier of personalized therapies. “[Personalized medicine] is permeating the cancer field. It is coming every place else,” said PMC Board Member Stephen Eck, vice president of oncology for **Astellas Global Pharma Development**, and a speaker at the briefing. The briefing was also mentioned as the first item in the “Happening Today” section of the **Politico Pulse** daily health news update and in **The Hill’s Healthwatch Blog**.

A May report from the **International Business Times** discussed the opportunity personalized medicine presents for medical device manufacturers and references the *Case for Personalized Medicine*.

Discussing controversies surrounding CER and how they could be mitigated should accountable care organizations (ACOs) become standard-bearers that use research to match treatment to patient, PMC Public Policy Director Amy Miller was quoted in May by **Inside Health Policy**. She pointed out that while the use of CER in decision-making would be less transparent if used

by ACOs than by CMS, conducting research that considers how genetic differences among people affect their response to treatments will still be needed, regardless of what body ultimately makes coverage decisions.

Modern Healthcare picked up remarks by PMC President Edward Abrahams at a seminar hosted in May by **RTI International** where he discussed barriers to personalized medicine including an unclear regulatory process, an outdated

reimbursement system, and a health care workforce that requires additional education in patient-centered medicine. “Even when there are products on the market which represent breakthroughs, they’re not necessarily likely to be used without massive marketing efforts,” he said.

In April, **GenomeWeb** wrote about goals for an FDA five-year plan to use CER to improve personalized medicine. Given the role of the PCORI in developing CER, the article noted PMC’s disappointment that none of the private sector members of the methodology committee have expertise in personalized medicine, and discussed our recommendation that the PCORI board of governors create an ad hoc advisory panel focused on personalized medicine.

In an April feature about personalized medicine for **R&D Directions**, PMC President Edward Abrahams said that what brings PMC members together is the fact that “the current system [is] broken [and]... patients deserve better.” Drawing from the *Case for Personalized Medicine*, the article mentioned personalized medicine success stories including FDA-approved personalized therapies such as Herceptin®, Erbitux®, and Gleevec®, as well as lesser-known efforts such as a collaboration between **Foundation Medicine** and **Novartis** to improve cancer genome testing, and an effort by **Johnson & Johnson** and **Pfizer** to use a specialized imaging biomarker to track an Alzheimer’s drug candidate.

Following the reintroduction of the PATIENTS Act by Senator Jon Kyl (R-Ariz.), in April, PMC Public Policy Director Amy Miller told **FDA Week**, “Aligning CER and personalized medicine needs to occur not only within HHS, but in PCORI” and that all stakeholders must come together to make research open and transparent.

