

The Personalized Medicine Coalition (PMC), representing a broad spectrum of academic, industrial, patient, provider, and payer communities, seeks to advance the understanding and adoption of personalized medicine concepts and products for the benefit of patients.

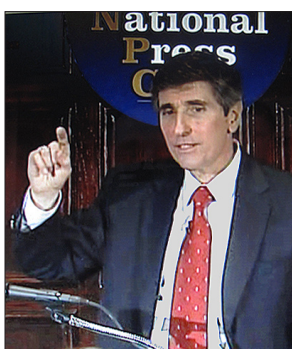
SPRING 2009

## Mayo Clinic CEO Offers New Vision for Personalized Patient Care

AT A TIME WHEN CONGRESS AND the administration are discussing changes in the healthcare system, Denis A. Cortese, M.D., president and CEO of the **Mayo Clinic**, offered a new vision for personalized healthcare in America at PMC's annual luncheon.

The key to fixing America's broken healthcare system is to measure the value of healthcare instead of its cost, Dr. Cortese told an audience of 100 officials from government, academia, and industry at the luncheon, which was sponsored by **Johnson & Johnson**. That's something the medical world understands instinctively, he said.

When he asks healthcare professionals and physicians the No. 1 problem they see in U.S. healthcare, "They all say, you know, we're spending an awful lot and we're not getting much for it," Dr. Cortese



Denis A. Cortese

said. "If you don't think you're getting what you paid for, that's a question of value."

Dr. Cortese defined value as the quality of the treatment a patient receives, divided by the cost to provide the treatment. Quality of treatment is measurable, he said. It includes measuring the safety and the effect of the treatment, as well as the patient's perception of whether he was helped.

Using a value equation will help America pay for results, while requiring doctors to comply with a process or treat a certain number of patients does not, he said. Right now, much of the most effective care the medical community provides is not being reimbursed.

"Some things are overpaid; some things are underpaid," Dr. Cortese said. "There is no connection at all" to the value of the service that is delivered.

Individualized medicine, including biomarkers for Tamoxifen and warfarin sensitivity and gene signature studies that are identifying other drug sensitivities in patients, can "significantly improve

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### *From the Executive Director*

## Obama's Election Creates Opportunities, Challenges for Personalized Medicine

THE ELECTION OF BARACK OBAMA AS President of the United States presents an unprecedented, perhaps not-to-be-repeated opportunity to advance the agenda for personalized medicine, change the way medicine is practiced, and improve healthcare.

President Obama wrote the first bill in Congress that identified the promise of personalized medicine. Among other things, that bill, which will be re-introduced in the 111th Congress, would create incentives to encourage the co-development of diagnostic and therapeutic

products, calls for funding to train a new workforce in genetics and genomics, establish a bio-banking database, and authorize an inter-agency workgroup to coordinate the government's policies so that they promote the new paradigm.

As president, Obama's horizon is much broader, but his interest in personalized medicine is likely to increase. He faces the central conundrum of healthcare: extend coverage to the fifteen percent of the American public that does not have health insurance, spur the



future research and development upon which patients depend, and at the same time cut systemic, exploding costs that reduce productivity in the economy.

Unless the president can simultaneously achieve these three competing goals, his healthcare plan is likely to face serious opposition. Americans, historically a "people of plenty," as the historian David Potter wrote more than fifty years ago, are nearly certain to reject any plan to which the label

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# Cortese Speaks at Annual Luncheon

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the value we deliver to patients,” he said. He urged the audience to embrace comparative effectiveness research, for which \$1.1 billion has been set aside in the new economic-stimulus law.

Many in the personalized-medicine world fear that the research will place too much emphasis on cost-containment rather than effectiveness, hurting personalized-medicine products.

But Dr. Cortese said that if the research is “done right,” it could drive the adoption of personalized medicine.

The culture of healthcare in America also must change to accommodate the explosive growth in knowledge that is accumulating thanks to our new understanding of molecular variation, among other things, Dr. Cortese said. Currently, little of the knowledge being accumulated in research is being incorporated into patient care. The stimulus package sets aside \$20 million in spending on health information technology.

“Health information technology plays a huge role in synthesizing large amounts of data, creating knowledge that will drive evidence-based decision making,” Dr. Cortese said.

The Mayo Clinic, the largest integrated, not-for-profit group practice in the world, is working to incorporate this technology for its doctors, he said. He emphasized the importance of interoperable computerized patient records and communication among the clinic’s 3,300 physicians, scientists and researchers and 41,000 allied health staff in determining the optimal treatments for individual patients.

Electronic patient records incorporate every aspect of an individual’s treatment, including details of doctor and hospital visits, treatments and results, he said. Where Mayo doctors used to pore

over patient records and medical books in its library to help them determine the best treatment in a case, they can now access the information by computer.

In addition, Mayo has built an electronic “learning system” for ten common diseases that doctors can use to find the latest medical research on the topic, leading experts on the disease at Mayo, and data about drug interactions. As more information becomes available on the ten diseases, it is added to the learning system.

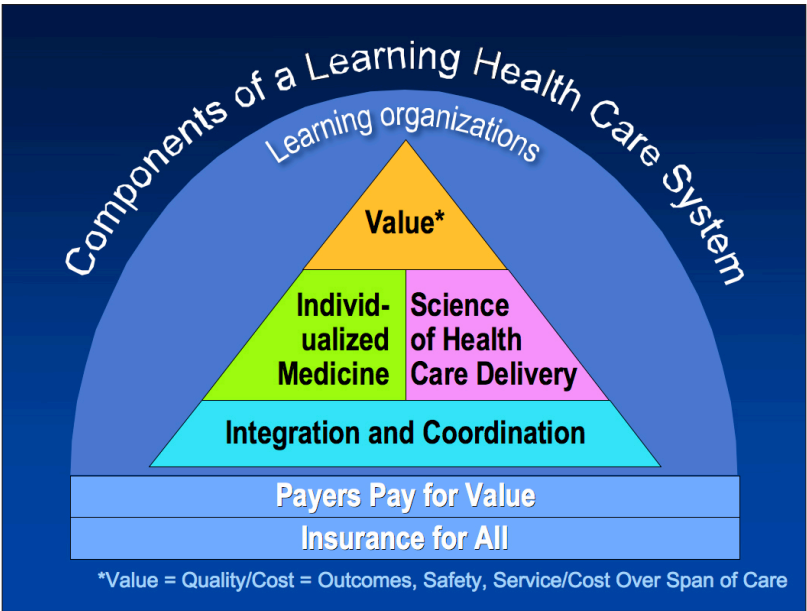
Dr. Cortese was introduced by Paul Stoffels, M.D., company group chairman, global pharmaceutical research and development, Johnson & Johnson.

Dr. Stoffels said that Johnson & Johnson also is committed to a personalized approach to medicine, using its broad expertise in healthcare research and development to develop targeted therapies for major diseases. Dr. Stoffels’ group is incorporating pharmacogenomics and biomarkers in drug discovery and drug development for treating the central nervous system and cardiovascular, metabolic, and infectious diseases, building upon earlier successes in treating HIV/AIDS.

Collaboration among industry, academia, providers, and payers has the power to fuel even faster develop-

ment and implementation of personalized medicine solutions, he said.

At Johnson & Johnson, the goal is “to use the latest science and technology and a patient-focused mindset to move away from a one size fits all approach to more targeted and personalized therapies,” Dr. Stoffels said. “This vision is aligned with Johnson & Johnson’s mission to care for the world, one patient at a time.”



*Mayo Clinic’s graphic depiction of a healthcare system in which value for the patient is the ultimate goal.*

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## Washington Begins Debates on Healthcare Reform and Comparative Effectiveness

**K**ANSAS GOVERNOR KATHLEEN SEBELIUS has been nominated to be Secretary of the Department of Health and Human Services (HHS), setting the stage for progress on the administration's healthcare reform goals. Nominations for the heads of the Food and Drug Administration (FDA), the Centers for Medicare and Medicaid Services (CMS), the National Institutes of Health (NIH), and the Centers for Disease Control and Prevention (CDC) are also in process.

Nancy-Ann DeParle, a former Clinton administration staff member in the Office of Management and Budget, will head the newly created White House Office for Health Reform. She has extensive Congressional relations experience, which will assist the White House's efforts to fashion healthcare reform in the coming months.

The healthcare reform debate has just begun, but Senate Finance Committee chairman Max Baucus, D-Mont. and House Ways and Means chairman Henry Waxman, D-Calif., say that Congress is committed to taking up a bill by August.

Meanwhile, the personalized-medicine world has been focused on the economic stimulus package, officially titled the American

Recovery and Reinvestment Act of 2009, which was signed into law on February 17.

The law contains \$20 billion for health information technology (HIT) and \$1.1 billion for comparative effectiveness research. While the HIT provisions are fairly well developed, the comparative effectiveness provisions are more vague. Of the total, \$400 million has been designated for comparative effectiveness research at NIH, and \$400 million is allocated for the Secretary of HHS to spend as she sees fit. Few parameters define how the money is to be spent.

An additional \$300 million of the comparative effectiveness money was allocated to the Agency on Health Research Quality, which in the past has funded systematic reviews of the literature and meta-analysis of published research. Since relatively little literature and research exists on individual variation, this could be problematic for efforts to better understand the benefits of targeted therapy for various diseases.

As a result of these provisions, the law has caused concern among proponents of personalized medicine. They fear



that rationing and reduced patient choice could result from poorly-designed comparative effectiveness research that is focused mainly on containing costs.

PMC helped persuade lawmakers to add legislative language to the conference report which articulates that the comparative effectiveness

funds are not intended to make medical care one-size-fits all, and that it should consider the relevance of "sub-populations." Now, we are actively working with key Hill staff to improve the comparative effectiveness research act and to prescribe how the comparative-effectiveness stimulus provisions could incorporate personalized medicine.

As federal agencies begin to spend the allocated funds, PMC will push them to follow the Congressional intent as well as the text of the law. PMC also is seeking an expansion of the personalized-healthcare initiative in the office of the Secretary of HHS to coordinate these efforts, in order to assure that comparative effectiveness research is personalized and evidence-based.

**Amy Miller**  
Public Policy Director

## Policy Updates

- PMC was asked to nominate a witness who could speak to the patient experience of personalized medicine for a Senate hearing on healthcare quality, and suggested Jeffery Gulcher, M.D., Ph.D., from **deCODE genetics**. Committee members and staff snapped to attention when Dr. Gulcher told them, "Personalized medicine probably saved my life." He described how he won his battle with cancer in part because of an early diagnosis through molecular-based diagnostics. Policy makers are getting the message that personalized medicine has the power to improve the quality of healthcare. We have asked Senator Barbara Mikulski, D-Md., who is leading the workgroup on healthcare quality for the Senate Committee on Health, Education, Labor and Pensions, to co-sponsor the Genomics and Personalized Medicine Act with Sen. Richard Burr, R-N.C.

- Separately, PMC has contacted Senator Richard Burr and Representative Patrick Kennedy, urging them to introduce an

updated version of the Genomics and Personalized Medicine Act in the 111th Congress. Both offices have indicated their interest in doing so and are seeking co-sponsors across the aisle.

**"Personalized medicine  
probably saved  
my life."**

*Jeffery Gulcher, deCODE genetics,  
in testimony at Senate hearing*

- On the regulatory side, the FDA has designated a workgroup to develop its 2005 concept paper on the co-development of therapeutics and diagnostics into a draft guidance. In 2008, Dr. Larry Lesko from FDA challenged PMC members to guide FDA in developing the guidance, and reiterated his call during a 2009 meeting with the clinical science committee.

PMC is responding with an outline of how the guidance should be structured and examples to highlight critical points. In its meeting with Dr. Lesko, the workgroup stressed the need for an open dialog between FDA and the community, as a great deal of time has passed since the concept paper was written. Members suggested that FDA revise the concept paper to reflect the latest scientific

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# Policy Updates

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findings and then discuss the draft with businesses, academics and others involved in the subject. This process would be an alternative to the more typical draft guidance that the public can only respond to in written comments, to which the FDA cannot reply except through a new draft guidance.

- Also at the FDA, Elizabeth Mansfield, Ph.D., has been named Senior Genomics Advisor, in the Office of the Chief Scientist. It is the first such post at the agency. She will coordinate efforts related to personalized medicine across the agency and will speak to the next meeting of the PMC policy committee on April 17.

- PMC circulated a letter to members of Congress regarding comparative effectiveness legislation in the economic stimulus bill. A copy is available on the public policy page of our web site at: [http://www.personalizedmedicinecoalition.org/sciencepolicy/public-policy\\_comparative-effectiveness.php](http://www.personalizedmedicinecoalition.org/sciencepolicy/public-policy_comparative-effectiveness.php). We are continuing to work with Congressional leaders to ensure that comparative effectiveness research recognizes that healthcare quality can often be improved by personalizing care.

- Dr. Mrunal Chapekar from the Technology Innovation Program at

the National Institute of Science and Technology spoke to members of the Public Policy Committee at the January 26 meeting. NIST wants to increase its work in the life sciences and is soliciting white papers to suggest where it can be most effective. PMC's response to the request for white papers is on our web site.

- SACGHS met on December 1-2 and on March 12-13. At the December meeting, PMC suggested priorities for the upcoming session of the group and updated SACGHS on our efforts. Because of our leadership in organizing standards in consumer genomics, PMC was asked to present at the March 12-13 meeting. Our presentation can be found on the SACGHS web site at: ([http://oba.od.nih.gov/SACGHS/sacghs\\_home.html](http://oba.od.nih.gov/SACGHS/sacghs_home.html)).

- At PMC's Public Policy Committee meeting on October 22, 2008, Shawn Bishop, Professional Staff Member, United States Committee on Finance, discussed S. 3408, the Comparative Effectiveness Research Act of 2008, introduced last year by Senators Baucus (D-MT) and Conrad (D-ND). PMC members suggested changes to the legislation that would incorporate personalized medicine and increase the quality of healthcare in the US.



## Comparative Effectiveness Research Provisions in the American Recovery and Reinvestment Act of 2009

Funds	Agency	Intended Use
\$300 million	AHRQ	Conduct research under Titles III and IX of the Public Health Service Act, title XI of the Social Security Act, and Section 103 of the Medicare Prescription Drug, Improvement and Modernization Act of 2003.
\$400 million	NIH	Conduct and support comparative effectiveness research.
\$400 million	HHS Office of Secretary	Speed research to assess comparative effectiveness of healthcare treatments. Research will compare clinical outcomes, effectiveness or appropriateness of treatment. Encourage use of clinical registries and other ways to electronically generate data on outcomes.  HHS may award grants and contracts to HHS or other government agencies and groups in the private sector with experience in achieving CER goals.
<b>Federal Coordinating Council</b>		The HHS Secretary will appoint 15 federal officials to the Council. At least half should be physicians or others with clinical expertise. The Council will include at least one person from AHRQ, CMS, NIH, FDA, VA, DOD and the National Coordinator for Health Technology.
<b>Conference report language</b>		The congressional report accompanying the bill notes that "clinical comparative effectiveness" has been changed to "comparative effectiveness;" CER money is not intended to be used to mandate coverage or reimbursement; a 'one-size-fits-all' approach to patient treatment is not the most medically appropriate solution to treating various conditions; and patient subpopulations should be considered in CER research. Conference reports may aid the administration in interpretation of a law.





## Personalized Medicine Can Bring Everyone Return on Investment, Study Finds

**P**ROVIDERS, PAYERS, AND INDUSTRY can all derive a return from an investment in personalized medicine under certain conditions, but patients benefit in all cases, according to a study presented at

PMC's conference on "Achieving a Return on Investment in Personalized Medicine" in January.

"Personalized medicine facilitates better care and lower costs, and has the potential

to benefit every stakeholder in the U.S. system—most importantly, its patients," said Paul Keckley, Ph.D., Executive Director of the **Deloitte Center for Health Solutions**, which conducted the study presented at the conference.

The conference and study were the first to focus on the issues faced by industry and policy officials when developing sustainable business models for personalized medicine. It explored the gaps that exist in the business models that prevent the level of investment needed to make personalized medicine a reality, as well as the role government could play. The conference was sponsored by **Affymetrix**.

### Framework for ROI

The study developed a framework for calculating ROI by examining case studies for two specific situations: altering the course of therapy (exemplified in the study by **Genomic Health's** breast-cancer assay

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*Top: PhRMA president and CEO Billy Tauzin talks about personalized medicine at PMC's January conference, "Achieving a Return on Investment in Personalized Medicine." Above: Paul Keckley, executive director of the Deloitte Center for Health Solutions, presents a study on the return on investment in personalized medicine.*





*Audience participants at the ROI conference, top to bottom: Ryan Phelan, founder of DNA Direct; Wayne Rosenkrans, Ph.D., PMC president and chairman, and a distinguished fellow at the MIT Center for Biomedical Innovation; and Henry Zheng, Ph.D., director for operations at the Center for Personalized Health Care at Ohio State University.*

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Oncotype Dx) or introducing a new targeted therapy (Genentech's Herceptin). A review of 300 studies revealed that everyone gains a positive return under certain conditions, but only consumers benefited consistently, a finding that was confirmed in Deloitte's meta-analysis, which analyzed 75 studies in greater depth.

Payers fared the least well in the study, due in part to the study's assumption that not all insurers would agree to cover the two products, and the fact that there is high turnover among health-plan customers. That would mean that the payers who covered the cost of the treatments might not benefit from the fact that the patients' health improved, since many of those patients would switch plans.

As a result, payers who covered personalized-medicine therapies received only a marginal benefit, and then only after six years, the study found.

## Issues with the Study

A number of attendees took issue with the study. Felix Frueh, Ph.D., Vice President of Research and Development for Personalized Medicine at **Medco Health Solutions**, said the study missed an essential point: Payers will save money by not having to pay for unnecessary treatments. What's important, he said, is to know how ROI scenarios compare or contrast to business models currently in use.

"Everyone agrees that the development of a new therapy, whether personalized medicine or not, should benefit the patient, generate revenue for the pharma/biotech sector, and cost the payer money," Dr. Frueh said. Dr. Frueh also said that Deloitte considered only a small portion of the peer-reviewed literature available, and did not make its models available for analysis. There is no way to see the parameters that were considered, the assumptions that were made, and the weights assigned to them, he said.

David King, president and chief executive officer for **LabCorp**, said it would have made sense to ask employers who

self-insure their companies for an opinion, since 70 percent of healthcare is covered by employers, not insurance companies.

## Employers Benefit

"Unlike insurance companies, employers do see immediate benefit from proper treatment with drugs such as Herceptin—cost savings plus a healthier employee population," he said. "When Deloitte says that it is not clear that the payers benefit in the near term, we should be clear that if employees live longer and are healthier, the employer (the true payer in most cases) in fact benefits immediately, regardless of which insurance plan administers the employer's health plan at a given time."

Joanne Armstrong, M.D., M.P.H., senior medical director for women's health at **Aetna**, also raised questions. "It is described as the ROI for targeted therapies, but it is actually a description of one approach to model a return on investment for specific technologies. It is important to not generalize the findings to the whole field of personalized medicine. It does underscore the need for data demonstrating the value of personalized medicine technologies."

## Empirical Data Lacking

Dr. Keckley said that Deloitte was unable to review a broader sample of personalized-medicine technologies because empirical studies that had control groups and sufficient outcomes data for other products such as warfarin sensitivity tests and Gleevec were not available. Empirical studies on employer-insured plans were also lacking, he said.

"The fact is that a database for ROI does not exist," Dr. Keckley said.

Although many products were not included in the meta-analysis because they lacked sufficient data, Deloitte found that they would likely benefit patients and reduce costs, based on its survey of the literature.

The study found that as a so-called "disruptive" healthcare innovation—one that challenges the status quo—personalized

*"Personalized medicine facilitates better care and lower costs, and has the potential to benefit every stakeholder in the U.S. system—most importantly, its patients."*

*Paul Keckley, Deloitte Center for Health Solutions*



medicine is likely to be adopted slowly without a better understanding of the promise, sustainability, and value of adopting it. More empirical studies will be needed to document the clinical value of personalized-medicine products, and government support such as tax breaks for “disruptive” business models that support personalized medicine would also be helpful, the study concluded.

## Comparative Effectiveness

In a presentation at the luncheon, Billy Tauzin, president and CEO of **PhRMA**, the trade association of the pharmaceutical industry, said that it’s important to figure out which drugs are most effective. But to advance the cause of personalized medicine, cost should play no part in the decision about which therapies are most efficacious.

In afternoon sessions on policy, government speakers said there are risks for personalized medicine as healthcare reform is taken up in the current cost-cutting environment.

Francis Collins, former director of the National Human Genome Research Institute, cautioned the audience to pay attention to the federal goal of identifying the optimal treatment or treatments for different



*“We should be clear that if employees live longer and are healthier, the employer (the true payer in most cases) in fact benefits immediately, regardless of which insurance plan administers the employer’s health plan at a given time.”*

*David King, LabCorp*

diseases, known as “comparative effectiveness.”

The United States has invested billions of dollars in basic science but very little in evaluating the outcomes of various treatments, said Janet Woodcock, director of the Center for Drug Evaluation and Research at FDA. As a result, the data do not exist to compare the effectiveness of various treatments. Rigorous

scientific evaluation of new products is needed, she said, not simply comparing costs. The notion of “eliminating waste” in the medical system also poses risks, she said. “Most care labeled waste is not uniformly useless, but the cost outweighs the benefits,” she said. “Doctors want to do these things because they help some people.”

President Obama has since signed into law an economic-stimulus package that includes \$1.1 billion for comparative effectiveness research [see Policy Update for more information].

Dr. Collins also predicted that over the next eight years, the science of personalized medicine will continue to advance, and costs will continue to fall. Researchers likely will have identified most of the heritable risks for common diseases, and a complete genome sequence will cost \$1,000, down from the current low of \$5,000, he said.



*Top, left to right: James Utley, vice president and medical director of the commercial business division at Coventry Health Care, talks about how payers decide whether or not to cover a treatment. Paul Keckley, Aiden C. Power, vice president and global head of molecular medicine for Pfizer Global Research and Development, and Randy Scott, executive chairman of Genomic Health, also participated in the panel, which offered perspectives from different parts of the personalized-medicine world about their return on investment. Bottom: Francis Collins, former director of the National Human Genome Research Institute, and Janet Woodcock, director of FDA’s Center for Drug Evaluation and Research, discuss federal policy on personalized medicine.*

**Elizabeth Schwinn**

## From the Executive Director

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“rationing” can be applied. Indeed, a plan that does not promise improved overall quality of care, offer an expectation that new discoveries will lead to better medicine tomorrow, and lower systemic costs is unlikely to garner sufficient public support to be enacted.

The only way to square the circle of increasing the quality and supply of health-care while simultaneously cutting costs is to deploy new technologies and new approaches that introduce efficiencies in the system while also emphasizing prevention, which will keep people healthier and also reduce the costs of chronic care. This is why during the recent presidential campaign, building on HHS Secretary Michael Leavitt’s generally unfunded initiative, President Obama

supported investing in health information technologies. Since the election, President-elect Obama has included \$19.2 billion in the economic stimulus package for health information technologies, money that could help build an infrastructure for more rational decision-making in medicine.

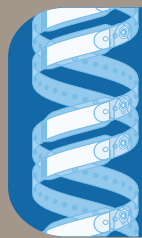
This is why the PMC’s agenda of increasing investment in translational research, creating a friendlier regulatory environment that facilitates the co-development of diagnostic and therapeutic products, and putting in place a reimbursement system that supports personalized-medicine products is all the more compelling.

We know that without more examples of widely used drug-diagnostic products, risk-

averse skeptics will not support reconstructing health systems around the world along these lines. Therefore, we will propose that government act sooner rather than later to establish clinical trials that would determine whether personalized approaches to care can produce better patient outcomes while reducing costs.

In brief, the principles of personalized medicine, including getting the treatment right the first time and preventing illness when possible, are the only way Obama’s health plan can achieve everything the American public wants.

**Edward Abrahams**  
*Executive Director*



# Personalized Medicine

[www.futuremedicine.com](http://www.futuremedicine.com)

## Free Subscription for PMC Members

## Consumer Genomics Industry Strives for Uniform Standards

**T**HOUGH LAUNCHED LESS THAN TWO YEARS AGO, GENE-SCAN information services that consumers can buy directly over the Internet have received more media attention than any other personalized-medicine product. Because of our educational mission and ability to convene a diverse cross-section of businesses and nonprofit organizations interested in personalized medicine, consumer genomics companies asked PMC to organize a discussion to help them address concerns regarding their products. One unintended consequence of gene-scan information services is that more Americans are now aware of the role genes play in health and the science of probability.

During a conference in December on consumer genomics hosted by the Personalized Healthcare Initiative of the office of the Secretary of Health and Human Services, representatives from **23andMe**, **deCODE genetics** and **Navigenics** agreed to develop standards for this new industry under the leadership of PMC.

The three gene-scan companies have reached consensus on some science standards (such as standardizing the calculation of individual risk), and have identified points of disagreement that will require the federal government to set standards (for example, the point at which a SNP is considered to be adequately validated). The results were presented during a consumer genomics event hosted by the CDC and

the NIH. Information on the science standards work can be found on our web site at <http://www.personalizedmedicinecoalition.org>.

The gene-scan companies and **DNA Direct**, which provides genetic guidance and counseling to consumers and others, recognize that subjective standards (such as what types of information they should give customers) must also be defined. Although scientists and federal regulators have cautioned customers about these products, patients and healthcare providers have not expressed their views. In an effort to address this gap, PMC co-hosted a roundtable with George Washington University titled *Consumer Genomics: Listening to Patients and Providers*.

The event drew participants from a number of patient groups and representatives of healthcare providers, including the **National Alliance for Hispanic Health**, Genetic Alliance, and the Alzheimer’s Association. The groups said they saw the potential of these products to improve healthcare for their constituencies, but expressed concerns about the value of the tests as well. Their views will help shape a consumer guide to the products.

Where this discussion will lead the industry is an open question. At the conference, Ryan Phelan of DNA Direct said to “expect the unexpected” in terms of the outcomes these products can have for consumers.





# Leaders in Personalized Medicine Join Forces for PMC

**D**RIVEN BY A DESIRE to accelerate the adoption of personalized medicine, seven leading proponents have formed the Friends of the Personalized Medicine Coalition to further advance personalized medicine and advise the Coalition at a critical time in its history.

The group has been created just as President Obama's election has made healthcare reform a top national priority.

That gives PMC an opportunity to see that the principles of personalized medicine—the right treatment, for the right patient, at the right time—are incorporated into the framework of the nation's healthcare system. If it succeeds, the United States will once again become a world leader in medical care, said Raju Kucherlapati, Ph.D., of Harvard Medical School, who chairs the new group.


"The implementation of personalized medicine is going to revolutionize the practice of medicine and put our nation at the forefront of providing high quality healthcare," he said.

In addition to Dr. Kucherlapati, other charter members of the group include M. Kathleen Behrens, a general partner in RS & Co. Venture Partners IV, who was a member of President Bush's Council of Advisors on Science and Technology; Brook Byers, a senior partner at **Kleiner Perkins Caufield & Byers**; Michael Goldberg, a partner with **Mohr Davidow Ventures**, Marcia A. Kean, CEO of **Feinstein Kean Healthcare**; Mark Levin,

former CEO of **Millennium Pharmaceuticals** and founder of venture capital firm **Third Rock Ventures**; and Ralph Snyderman, M.D., president emeritus of **Duke University Health System**.

"It's no accident that this group has formed as healthcare undergoes a major

transformation catalyzed by new technologies," said Ms. Kean.

"Each of us has been a pioneer in our respective fields and can see the special opportunity that PMC has," she said. "We're here to support PMC and see that it has the appropriate resources to maximize its impact, and we are very dedicated to its success. Once a Friend, always a friend." 



## **Charter of the Friends of the Personalized Medicine Coalition**

*Whereas....* We believe that a revolution in health care is underway, known as personalized medicine, catalyzed by rapidly-emerging information from life sciences research and the availability of sophisticated information technologies;

*Whereas....* We are witnessing the initial clinical benefits of personalized medicine strategies and products, in the form of a new generation of molecular diagnostics and targeted therapeutics that can improve efficacy and reduce adverse side effects;

*Whereas....* We wish to accelerate the adoption of personalized medicine to improve individual patient outcomes as well as the overall effectiveness of the health care system;

*Whereas....* We view the mission of the PMC -- to educate federal and state policymakers and private sector healthcare leaders about personalized medicine, helping them understand the science, the issues and what is needed for the positive evolution of personalized medicine -- as critically important;

*Whereas....* The PMC has demonstrated its unique role in the field by encompassing all sectors of the biomedical enterprise within its membership, and effectively framing the debate around the key obstacles to adoption of personalized medicine;

*Whereas....* We believe that, in addition to the membership role played by each of our organizations in the PMC, there is an opportunity for individual leadership and commitment to advance the goals of the PMC;

*Hereby Be It Resolved that....* We as individuals establish and support the Friends of the Personalized Medicine Coalition, and invite our colleagues and peers to join us.

### **Friends of the Personalized Medicine Coalition**

Raju Kucherlapati, Chair  
M. Kathleen Behrens  
Brook Byers  
Michael Goldberg  
Marcia A. Kean  
Mark Levin  
Ralph Snyderman

January 1, 2009

## Clinical Science Committee Appoints New Co-Chair

• PMC's Clinical Science Committee stepped up its advisory and oversight pace at its February 12 meeting in Washington, D.C., as committee chair Charis Eng, M.D., Ph.D., chair and director of the Genomic Medicine Institute at the **Cleveland Clinic**, introduced her new co-chair, Jeffrey Cossman, M.D. Dr. Cossman is Chief Scientific Officer of the **Critical Path Institute**.



Jeffrey Cossman

The meeting focused on FDA's plan to regulate co-developed drugs and diagnostics—products that combine a diagnostic test and therapeutic treatment in one. Lawrence J. Lesko, Ph.D., director of FDA's Office of Clinical Pharmacology at the Center for Drug Evaluation Research,

summarized FDA's experience to date with such drugs and diagnostics. "Co-developed" is something of a misnomer, he said, since the diagnostics may be developed before or after a drug goes to market.

Robert Yocher, vice president of regulatory affairs for **Genzyme Genetics** and chair of PMC's workgroup on co-developed drugs and diagnostics, which is drafting proposed regulations at the FDA's suggestion, said the workgroup is coordinating input from PMC members and even outside groups from a variety of perspectives, including the diagnostic, pharmaceutical, laboratory, and payer communities.

Members discussed other PMC communications, including a new draft of the *Case for Personalized Medicine*, PMC's signature document, which will be published May 1.

The committee also decided that an article in the *Annals of Internal Medicine*, which found that PGx testing for Warfarin was not cost effective, merits a response.

Copies of Dr. Lesko's slides and an outline of the PMC workgroup's effort on co-developed drugs and diagnostics are available on our website at <http://www.personalizedmedicinecoalition.org>.

• At the Clinical Science Committee meeting on October 22, 2008, Dr. Linda Bradley of CDC's EGAPP, spoke with PMC about how EGAPP develops their recommendations. She said she recognizes that the personalized-medicine community is concerned that EGAPP studies usually conclude that "there is not enough evidence to recommend the test" and suggested that PMC outline how the process could be improved.

## New Members

5AM Solutions, Inc.

AdvaMed  
(Advanced Medical Technology and Trade Association)

Arrowhead Publishers and Conferences

Axial Biotech, Inc.

BD

Bristol-Myers Squibb Company

GlaxoSmithKline

Growing Company Solutions, Inc.

H. Lee Moffitt Cancer Center & Research Institute

Helix Health LLC

The Jackson Laboratory

Lemhi Ventures

Life Technologies Corporation

Merrimack Pharmaceuticals, Inc.

Molecular Image, Inc.

National Brain Tumor Society

Premier Source

Prognomix Inc.

Scientia Advisors

Sequenom Center for Molecular Medicine

Technic Solutions LLC

Townsend and Townsend and Crew LLP

## SAVE THE DATES...

... for PMC's upcoming community events in:

Research Triangle Park, N.C.—May 11

San Diego—June 9

San Francisco—June 12

More Details Coming Soon



## HHS Secretary Leavitt Receives PMC's Leadership Award

**T**HE PERSONALIZED MEDICINE COALITION HAS GIVEN MICHAEL O. Leavitt its Leadership in Personalized Medicine Award for his leadership in engaging government to advance personalized healthcare on a national scale.

The annual PMC award recognizes the contributions of a visionary individual whose actions in science, business or policy have advanced the frontier of personalized medicine.

Leavitt, the former Secretary of Health and Human Services, "provided extraordinary leadership in the recognition of the importance of personalized medicine in health care," said Raju Kucherlapati, Ph.D., of the Harvard Medical School, who nominated the secretary for the 2008 award. "He is intensely focused on making health care more transparent in quality and price, and reducing the time and expense of bringing safe and effective drugs to market."

Leavitt served as HHS Secretary from 2005 through 2008. In that post, he established an office within HHS that communicated to a wide range of agencies the implications of personalized medicine, and the need to integrate science with information technology to realize its promise. Among other activities, he proposed that HHS create a network of data repositories across the country to enable more organized research in personalized medicine. His presence at annual PMC meetings generated news coverage of the issues and increased their visibility. He also showed himself willing to work across party lines to further important scientific goals.



*Raju Kucherlapati of Harvard Medical School speaks with then-Health and Human Services Secretary Michael O. Leavitt at a conference in November. Dr. Kucherlapati nominated Mr. Leavitt for PMC's annual Leadership in Personalized Medicine award.*



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# PMC in the Press

In an in-depth article on comparative effectiveness, **Pharmacogenomics Reporter** said that some industry stakeholders, researchers, and patient advocates are urging the use of pharmacogenomic tools to focus on improving patient outcomes and not solely on reducing healthcare costs. “The PMC is not opposed to comparative effectiveness. We just hope that it’s done right,” PMC executive director Edward Abrahams told the publication. The article also quoted from Mayo Clinic president Denis Cortese’s and Johnson & Johnson group chairman Paul Stoffels’ speeches at PMC’s annual luncheon. Both speakers said that finding out which treatments are most effective will help personalized medicine.

In the April issue of **Personalized Medicine**, a journal published in the United Kingdom and distributed worldwide, PMC executive director Edward Abrahams wrote that many in the United States think that comparative effectiveness research is a way to reduce the cost of healthcare. President Obama recently signed the economic-stimulus law, which includes \$1.1-billion for the government to conduct such research, under which the outcomes of different treatments for particular diseases are compared to see which works best. Abrahams cautioned that if only cost, rather than effectiveness, governs the research, access to many personalized-medicine therapies could be limited and promising research could be slowed. The government should employ a sophisticated determination of effectiveness, he said. Many targeted therapies are not effective for the general population, but they work very well for certain groups.

**The Pink Sheet** reported in February that Randy Scott, a speaker at a Personalized Medicine Coalition’s conference, said that the drug and diagnostic industries should partner but not merge. Scott, founder and executive chairman of the diagnostic firm **Genomic Health**, said it will be best for all if the two industries remain separate. “The diagnostic industry will represent a check on the pharmaceutical world, and the pharmaceutical world will represent the check on the diagnostic world. That’s healthy for the system.” Scott spoke at PMC’s conference on return on investment in personalized medicine at the end of January. **Health News Daily** also cited Scott’s remarks in a separate article in February about a webinar hosted by Elsevier Business Intelligence.

**InsideHealthPolicy** reported in February that money for comparative effectiveness research in the stimulus bill could hurt personalized medicine, according to Janet Woodcock, who spoke at PMC’s January conference on the return on investment in personalized medicine. FDA has raised concerns with the White House that the economic stimulus package moving through Congress would boost comparative effectiveness research without enough focus on personalized medicine, according to agency drug center director Janet

Woodcock, who spoke at the conference. Woodcock said the FDA is not opposed to comparative-effectiveness studies, but expressed concern that the emphasis on cost-effectiveness could lead to a “one-size-fits-all” approach that denies patients care.

**The Pink Sheet** reported in February that private payers may have difficulty realizing a positive short-term return on their investment in covering a targeted breast cancer therapy like Genentech’s Herceptin, according to a study by the **Deloitte Center for Health Solutions** that was presented at PMC’s January conference on the return on investment in personalized medicine. In the study, Deloitte assessed the barriers and incentives for adopting personalized medicine, concluding that the “high costs of personalized medicine therapies make it difficult for payers to recoup their early reimbursement of a companion-targeted therapy and its associated adverse events and risks.” **GenomeWeb Daily News** also issued a report on PMC’s conference in January.

In a December article titled “Personalized Medicine: The Future of Healing,” **Newsweek** magazine describes a new paradigm for medicine that exploits the genetic variation among individuals, and shows describes in a graphic how it has been effective in stopping HIV, targeting cancer, and treating depression. The Personalized Medicine Coalition is cited as a source for the article.

The **Wall Street Journal** reported in a December feature on Emory University’s predictive health assessment, which looks at genetic markers and other measures to figure out what risks a patient has for various diseases. The Personalized Medicine Coalition’s web site is cited as a source for more information on personalized and predictive medicine.

**Associated Press** reported in November that personalized medicine is likely to get a major push from the incoming administration of President-elect Barack Obama. As a senator, Obama introduced legislation to coordinate the sometimes conflicting policies of government agencies and provide more support for private research. The article quotes PMC executive director Edward Abrahams saying, “It would be very helpful if you could get Medicare and FDA talking to each other. Right now they really don’t communicate very well, and they have different agendas. The federal government is not coordinated around removing the barriers to personalized medicine.”

**GenomeWeb Daily News** reported in November that the Personalized Medicine Coalition has awarded its Leadership in Personalized Medicine Award to former HHS secretary Michael Leavitt. Leavitt’s leadership “has raised the level of awareness about the promise of personalized medicine to heights it would not have reached otherwise.”

