

FROM THE PRESIDENT

An Agenda for Personalized Medicine that includes robust plans for 2014

SCIENCE POLICY BRIEF

Phyllis Frosst discusses PMC's agenda for Science Policy

GUEST COLUMN

The Global Reach of Personalized Medicine

The Economics of Personalized Medicine



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Education & Advocacy recently sat down with Professor C. Daniel Mullins, Ph.D., at the University of Maryland to get his views on issues in personalized medicine (PM), including questions regarding value, pace of progress, and patient engagement.

When we talk about PM, why does it always seem to be more expensive?

PM by its very nature reflects the fact that there is no such thing as "one size fits all" when it comes to health care. As a result of tailoring health care and treatments to individuals, the initial costs go up; however, if PM helps to assure that individuals receive the best personalized care, then future costs will go down as we eliminate the wasteful

spending associated with "trial and error." Historically, before PM aided physicians in making treatment recommendations, patients would be placed on one medicine to see if it would work and then subsequently switched to another treatment if the first one failed to work. Not only does that represent wasteful spending on the initial ineffective therapy, but it also places the patient at risk

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An Agenda for Personalized Medicine

BY EDWARD ABRAHAMS, PMC PRESIDENT

Dedicated to promoting the understanding and adoption of personalized medicine to benefit patients and the health system, this year PMC celebrates its tenth anniversary.

With a strong board that represents the diversity of its 220 members from across the health care system and the globe, a new chair, William Dalton, CEO of M2Gen and Director of the Personalized Medicine Institute at the Moffitt Cancer Center, and a healthy balance sheet, PMC is well positioned to:

- Advocate for public policies that support personalized medicine's development through scientific, regulatory, and reimbursement-related activities.
- Educate and build a personalized medicine-informed health care system.
- Form consensus among personalized medicine stakeholders to promote common goals.

To realize this bold agenda with its mix of educational and advocacy initiatives, PMC plans to concentrate on three overarching programs.

First, with our partners the American Association of Cancer Research and Feinstein Kean Healthcare, we will continue to develop an "innovation agenda" that emphasizes practices and policies between the science and the patient that will improve cancer

research and care. These include patient engagement, evidence generation, and a commitment to a learning-based health care system that utilizes health information technology, all of which, we assert, will improve patient outcomes while increasing efficiency and maximizing value. A conference that examines and analyzes these ideas is planned toward the end of the year.

Second, recognizing that personalized medicine depends on our willingness and ability to pay for both diagnostic and therapeutic products, PMC will focus on reimbursement issues this year. The Coalition's Tenth Annual State of Personalized Medicine Luncheon Address at the National Press Club in Washington, D.C., is scheduled for March 13 with guest speaker Patrick Conway, Director and Chief Medical Officer of CMS. He will share with us his

vision of personalized medicine and answer questions about developing payment policies. To provide a better understanding of the evolving legal and regulatory landscape for reimbursement, later this year the Coalition will issue an updated landscape analysis that defines the field, as did our white paper, *Pathways for Oversight of Diagnostics*, published last year. The new essay will set the stage for future discussions of evidentiary standards used to make decisions about paying for and adopting personalized medicine products. Along with our partner, BIO, PMC envisions a defined process that diagnostic companies and payers can agree to which outline the evidentiary standards used for coverage of molecular diagnostic tests before they are developed, thereby eliminating much of the confusion and contention that

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Public Policy Paves the Way For Personalized Medicine

THE PERSONALIZED MEDICINE COALITION submitted comments to the Senate Finance and House Ways & Means Committee staff on their bipartisan, bicameral proposal to fix the Sustainable Growth Rate (SGR) and Medicare Physician Fee Schedule.

PMC believes that the committees need to be mindful of the significance of personalized medicine and of patient-centered health care as they move the bill forward, and that new payment models must be careful not to unintentionally undermine the development of and patient access to innovative personalized treatments.

“The evolution and growth of personalized medicine and the pace of medical innovation are continually changing the current standards of patient care. Alternative payment models should be designed to give providers the ability to tailor care to individual patients and create incentives for patient engagement...” stated in PMC submitted comments.

You may view PMC’s full comment letter on our website.

FDA Publishes Guidelines on Mobile Health Applications

The FDA issued the Mobile Medical Applications Guidance for Industry and Food and Drug Administration Staff, a document which explains the agency’s oversight of mobile medical

apps as devices and its focus only on the apps that present a greater risk to patients if they do not work as intended and apps that cause smartphones or other mobile platforms to impact the functionality or performance of traditional medical devices.

Mobile medical applications are intended to help people manage their own health and wellness, promote healthy living, and gain access to useful information when and where they need it. According to industry estimates, 500 million smartphone users worldwide will be using a health care application by 2015, and by 2018, 50 percent of the more than 3.4 billion smartphone and tablet users will have downloaded mobile health applications (<http://www.research2guidance.com/500m-people-will-be-using-healthcare-mobile-applications-in-2015/>). These users include health care professionals, consumers, and patients.

A full version of the FDA document can be found on its website.

Reform Update: PCORI to fund \$93.5 million for clinical research network

The Patient-Centered Outcomes Research Institute (PCORI) approved \$93.5 million to support 29 clinical research data networks that together will form a new resource known as PCORnet, the National

Patient-Centered Clinical Research Network. PCORI envisions PCORnet to be a secure, national data network that improves the speed, efficiency, and use of patient-centered comparative effectiveness research (CER). By integrating data available in the 29 individual networks, PCORnet aims to support a range of study designs by providing access to a large amount of diverse, nationally representative health information to researchers and clinicians. This effort will reduce the time and effort needed to launch new studies and focus research on questions and outcomes especially useful to patients and those who care for them.

A Partial Victory on MAAAs

In December 2013, the Centers for Medicare and Medicaid Services (CMS) issued new and reconsidered Clinical Laboratory Fee Schedule (CLFS) Test Codes and final payment determinations for 2014. Of note, tests classified as Multianalyte Assays with Algorithmic Analysis (MAAA) can now be covered if the Medicare contractor determines the code is payable. The following rationale underlies this decision:

“Medicare pays for diagnostic tests that are used by the patient’s treating practitioner to determine the appropriate course of treatment if all

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A Science Policy Portfolio

BY PHYLLIS FROSST, PMC VICE PRESIDENT, SCIENCE POLICY

Now, more than 10 years into the genome era, our challenge remains how to translate genomics and other new discoveries in science and technology into better medicine.

This year, the Personalized Medicine Coalition will contribute toward that goal by creating a new portfolio for science policy to supplement our work in public policy and expand PMC's scope.

As PMC's newest staff member and Vice President of Science Policy, I have the opportunity to use my experience as a research scientist and a policy advisor at the National Institutes of Health to help PMC engage the issues that will impact the pace of progress in personalized medicine.

Our implementation plan for Science Policy will evolve through interactions with our members and the PMC's Science Policy Committee, chaired by Amy Abernethy, Ph.D., M.D., Director of the Duke Center for Learning Health Care. The Committee evaluates emerging applications of science and technology in personalized medicine and serves as an advisory resource for the PMC. Since member insight and advice will help guide our path as we develop PMC's science policy agenda, we encourage you to join, participate in our meetings, and contact me personally.

Among other things, the Committee will review the 4th edition

of PMC's *The Case for Personalized Medicine*, revised during the winter of 2014.

The PMC science policy portfolio will focus on NIH funding and policy, clinical trial design, health IT, next-generation sequencing regulatory science, bioethics, and the practice of medicine.

Clinical Trial Design

As genomic data become more prevalent, clinical trials must evolve, based on a more thorough understanding of the genetics that underlie disease and the effects of small molecules and biologics. Patient populations can be divided into ever smaller cohorts (defined by their molecular attributes), offering opportunities for innovative and adaptive clinical trials that have the potential to accelerate discovery and lower costs.

NIH Funding

Working closely with the FDA, NIH has led the charge toward personalized medicine. I was fortunate to have spent four years in the policy office of Dr. Francis Collins, who was then Director of the National Human Genome Research Institute (NHGRI), and is now the Director of the NIH. He has embraced an ambitious policy agenda, which includes working with federal agencies, the private sector, and Congress to advance personalized medicine. But unfortunately, funding for the NIH has suffered since his tenure as NHGRI Director, in ways that could have long-reaching impacts. Although Congress has increased NIH funding in FY2014 to almost \$30 billion, \$1 billion more than last year, that figure is \$714 million less than before sequestration cuts took place.

As genomic data become more prevalent, clinical trials must evolve, based on a more thorough understanding of the genetics that underlie disease and the effects of small molecules and biologics.

Health IT

At NHGRI in 2004, the cost of a single genome sequence was close to \$5 million. In the decade since, the promise of genomic medicine has made its way into the clinic. With Illumina's recent announcement of a \$1,000 genome sequence, we are another step closer to the dreams of the genome project pioneers. But massive sequencing capacity represents only the first stage of improving human health through genomic tailored diagnosis and treatment selection. With more than 90 percent of American physicians using electronic health records, PMC will help make the case for society

to leverage health IT investments in order to address ongoing concerns, including interoperability, data sharing and complex consent.

Conclusion

I have learned a lot about policy in the decade since the human genome project was completed. It is widely recognized that good policy is based on good science, evidence and data. But, to actually effect change, the facts need to work in concert with social norms, morals, values and personal responsibility.

This is to say that good science is necessary but not sufficient for policy

development, which requires the addition of sage judgment. Bottom line—the facts without a trusted source or a thoughtful understanding of their context in the broader society will often lead to an impasse, something proponents of personalized medicine need to avoid.

In this context, our goals for science policy at PMC are:

- Ground science policy work in the best science possible.
- Become a trusted partner to policy-makers and to personalized medicine's stakeholders.
- Engage in policy work that focuses on solutions and not simply on problems.

Public Policy Paves the Way For Personalized Medicine

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other Medicare statutes, regulations, and/or policy requirements are met. The above codes have been classified beginning in the 2013 CPT as Multianalyte Assays with Algorithmic Analysis (MAAA). However, we do not believe there is a single consistent definition for a MAAA that allows us to make a categorical determination of whether these codes constitute diagnostic laboratory tests. We will continue to consider each individual test that is classified by the CPT as a MAAA on its own merits," stated CMS documents.

The full Clinical Laboratory Fee Schedule can be found on CMS' website.

First FDA Authorization for Next-Generation Sequencer

The U.S. Food and Drug Administration approved four Illumina "next-generation" gene sequencing devices with the potential to make faster and more precise diagnoses. The marketing authorization for the first next-gen sequencer represents a significant step forward in the ability to generate information that will ultimately improve patient care. Francis

Collins and Margaret Hamburg called the decision a "landmark move that will help realize the promise of personalized medicine." But there are still many challenges ahead for personalized medicine, they said, including support for health care professionals in interpreting genomic data, reimbursement issues, the validation of new genomic discoveries so they can be incorporated into medical decision-making, and addressing relevant policy, legal, and regulatory issues.

Details of the approval can be found on FDA's website.

The Economics of Personalized Medicine

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for serious adverse outcomes. Those negative health outcomes represent additional wasteful spending that can be eliminated through more tailored treatment guided by PM.

Why has the adoption of PM been slower than what was predicted a decade ago?

Part of the reason that PM has been adopted more slowly than anticipated is because it is viewed as expensive. Consequently, many payers may be waiting for the cost to come down. In addition, many patients and their health care providers are uncertain about the value of PM, so there isn't yet a high demand for these novel approaches to care. As the value story evolves for PM and as patients and their health care providers become more comfortable with more personalized approaches to health care, the uptake will increase. In addition, as the cost of certain components of PM come down, the increased demand for PM will be reinforced.

What is the story around financing PM for prevention? Is it true that prevention doesn't make good financial sense in the short term?

That's exactly right! The increased costs of PM are viewed right away by payers while the downstream savings and elimination of wasteful spending may be months or even years down



C. Daniel Mullins, Ph.D., Professor and Interim Chair Pharmaceutical Health Services Research Department, University of Maryland School of Pharmacy.

the road. In fact, a good part of PM is about prevention, and it is hard for many patients and payers to fully grasp the cost savings of bad health outcomes that are avoided, precisely because they don't ever see them. In some ways, PM is like buying a security system—it's an investment in avoiding harmful things in the future.

What are the most common misconceptions about economics and PM?

Somehow people think that value in health care should mean cost savings, when in fact we often pay slightly more to get better health outcomes. The term "cost-effective" is not synonymous with "cost savings." Cost-effective health care purchasing refers to situations in which the money spent provides "good value for money." At times, we might save money, but sometimes we spend more because there is sufficient clinical value and improvements in quality of life to justify the additional amount spent. Not everything that improves health reduces wasteful spending in the future to fully compensate for the upfront cost of PM. Sometimes, PM provides dividends in terms of improved health rather than reduced wasteful spending. For example, patients may participate in PM that reduces adverse outcomes that impact the quality of their lives, but would not have led to hospitalizations.

As a result of tailoring health care and treatments to individuals, the initial costs go up; however, if PM helps to assure that individuals receive the best personalized care, then future costs will go down as we eliminate the wasteful spending associated with "trial and error."

What are your thoughts on the interplay between the roles of the federal government and the private sector in the adoption of PM?

Americans expect that the government should invest in the health of the nation. That means that in some form or another the government needs to reward companies that bring health-improving innovations to the marketplace. This can be done at the front end, by providing tax credits or other incentives for investment or it can be done at the time of purchasing, such as when Medicare, the Department of Defense, or Medicaid pay for innovative PM. Private insurers who are for-profit companies don't have the same incentives to think about population health over the long run, so the government needs to intervene in order to encourage continued innovations in PM.

What can you predict about the economic viability of molecular diagnostics in 2014 and beyond?

I don't know if it will happen in 2014 or later, but eventually the growth will

be exponential. As the cost of diagnostics comes down and their predictive ability or clinical utility rises, the value will increase tremendously. At that point, the demand will rise substantially. Another current concern is that many people are still worried about what will happen with their genetic information so they are reluctant to participate in any testing that they think could be used to deny coverage as a pre-existing condition. Some of the aspects of the Affordable Care Act could help to address these fears and promote greater use of molecular diagnostics in the future.

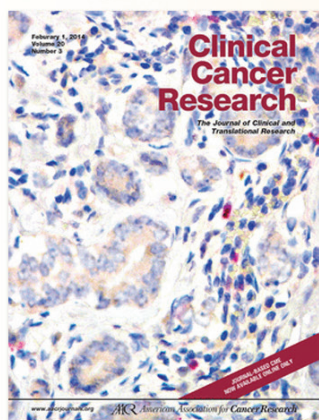
How much of a role will patients play in the adoption of PM?

Patient-centered care has re-emerged in recognition of the need for the patient to be the center of all health care. The next step is patient-centered outcomes research, whereby patients are actively engaged in helping to define the types of health outcomes that are most important to patients. When we listen to what is important to patients, we can tailor future research and PM

to meet their personalized needs and preferences. Otherwise, we run the risk of more wasteful spending in the future because patients are more likely to be non-adherent when they don't perceive that their treatments meet their needs. At the University of Maryland, we have developed a 10-step engagement framework for promoting patient centeredness along the research continuum from developing research ideas through conducting studies all the way to translation and dissemination. We believe that patient engagement in transforming health care research and practice will lead to greater adoption of evidence-based medicine, including PM.

How will comparative effectiveness research (CER) assist in the adoption of PM?

Here's where I think that the Agency for Healthcare Research and Quality (AHRQ) and the Patient-Centered Outcomes Research Institute have done a great job in starting to address patients' concerns. By promoting CER and patient-centered outcomes



Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress

Article Published in AACR's
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research, these institutions are encouraging researchers to answer health-related questions that people care about and understand. CER not only provides evidence on the comparative effectiveness of alternative approaches to managing disease, but it also recognizes the diversity of patients in America. PM can benefit from improved evidence that helps to address diversity in both the response to treatment as well as preferences for alternative treatment strategies.

Genome sequencing costs continue to decrease, but the argument is still valid that we don't know enough about the human genome to use that information effectively. How do we remedy the situation in a timely manner, as patients will soon be showing up at their doctor's office with their genomes sequenced?

We need to educate patients on what genetic testing can—and cannot—do for helping them to make more informed decisions. People think of

Many patients and their health care providers are uncertain about the value of PM, so there isn't yet a high demand for these novel approaches to care.

these tests as though it is a light switch and think that the bulb either goes on or off, but that's not usually how it works. Often, the test results are about probabilities rather than certainty; and people do not like uncertainty. Moreover, many people simply don't understand statistics. Patients want their doctors to tell them what is the best treatment and they want a test to predict with 100 percent accuracy. Unless genomic science gets closer to perfect predictions, we'll always have people who refuse to pay for them. For those who do show up at their doctor's office with their genomes sequenced, we'll need to simplify the clinical and statistical evidence so that it is meaningful to inform their treatment decisions.

What are the roles for post-hoc analysis of medical record data, and literature reviews in evidence generation for PM?

We're developing good methodological standards for such analyses. When people follow best practices, these studies contribute valuable information that may not be obtained from more traditional approaches such as randomized clinical trials. However, it is important for people to understand how to interpret the results of such studies and that includes their limitations. There are limitations to all studies, including randomized trials, so we need to look at all forms of evidence and synthesize evidence across studies.

Background

C. Daniel Mullins, PhD is a professor in the Pharmaceutical Health Services Research Department (PHSR) at University of Maryland School of Pharmacy.

His research focuses on pharmacoconomics, comparative effectiveness research,

patient-centered outcomes research, and health disparities. He has received funding from the Agency for Healthcare Research and Quality (AHRQ), National Institute on Aging (NIA), the National Heart, Lung, and Blood Institute (NHLBI), the Patient-Centered Outcomes Research Institute (PCORI), as well as pharmaceutical companies, the

insurance industry, and patient advocacy organizations. Prof. Mullins also serves as the Associate Director of the Center on Drugs and Public Policy at the School of Pharmacy and the Co-Editor-in-Chief of Value in Health.

Prof. Mullins received his BS in Economics from M.I.T. and his PhD in Economics from Duke University.

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Kathy Giusti, Ninth Annual Award Recipient

Recognized for her Leadership in Personalized Medicine

Remarks by Kathy Giusti

Wednesday, November 6, 2013

Following is an excerpt from an acceptance speech by Kathy Giusti, Founder and Chief Executive Officer, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium, who received PMC's 2013 Leadership in Personalized Medicine Award.

The award is given annually at the Personalized Medicine Conference at Harvard Medical School to recognize an individual whose contributions in science, business, and/or policy have advanced personalized medicine.

I am not a scientist, and I am not a clinician. I'm a businesswoman who at a young age happened to get diagnosed with a fatal cancer, and as a wife and the mother of a beautiful 1-year-old little girl, I said to myself, "Well, I think I've got to put in the best fight that I possibly can," and the only thing I really knew how to do was run a business. So I started



Edward Abrahams, left, PMC President, Kathy Giusti, Founder and Chief Executive Officer, Multiple Myeloma Research Foundation and Multiple Myeloma Research Consortium, and William Dalton, PMC Chair, CEO of M2Gen, and Founding Director of Moffitt's Personalized Medicine Institute.

the Multiple Myeloma Research Foundation back in 1998. There were two things that helped me, and one

was my training out of Harvard Business School and the discipline I had on writing very stringent business plans, and the second piece was that my job had been running worldwide operations for a major pharmaceutical company when I was diagnosed.

When I was diagnosed in '96, the five-year survival rate of Multiple Myeloma was 28 percent. When the doctors looked at me and said, "Kathy, we're really sorry, but you will live three years at best, and you need

When the doctors looked at me and said, "Kathy, we're really sorry, but you will live three years at best, and you need to quit your job in the pharmaceutical industry, and you really need to move East and just get close to your family again, because it's that bad," there was absolutely no reason to disagree with them.

to quit your job in the pharmaceutical industry, and you really need to move East and just get close to your family again, because it's that bad," there was absolutely no reason to disagree with them. I looked at the pipeline. There was nothing going on. There was no awareness, no funding, very few scientists.

We started the Foundation to focus on research and funding grants to bring scientists into the fold. While many foundations, thank goodness, have followed our lead and now are being trusted third parties supporting research and drug development, I can promise you in 1998, we were one of the first with research in our name.

When we first started getting the scientists funding and they started to come our way, we said there is no easy way for them to work together, even ironically, in a small cancer field. Myeloma sees only 20,000 new patients a year. We said we need critical mass. We cannot have fragmentation in the system.

What we decided to do was start a second foundation called the Multiple Myeloma Research Consortium (MMRC). Founded in 2004, the membership agreement at MMRC is a take-it-or-leave-it membership agreement, and we have 16 centers that work with us. The beautiful thing about it is if you do well in our clinical network, you get money and manpower. If you do not do well, we throw you out, and I have to say that transparency in metrics has helped us to really drive a wonderful, and tight ship.



Kathy Giusti delivers her Leadership Award acceptance speech at the Ninth Annual Personalized Medicine Conference.

Our progress includes more than 4,000 bone marrow biopsies banked, six drugs approved in the last 10 years, and myeloma patients are living more like seven, eight, or nine, rather than the three years I was given when I was diagnosed.

But the truth is, the more we started investing in the field and the more we started looking into genomics, we realized that myeloma is not one disease, but it's actually more like 10 subtypes. So it became very clear that our business plan was going to be a business plan focused on personalized medicine.

We decided that the first thing we were going to do in personalized medicine was to launch what we called the MMRF CoMMpass Study, where we would follow 1,000 patients

from that very moment of diagnosis, before they were treated. To date, the CoMMpass Study has enrolled 400 patients (just a note to say that we're already approaching 450!). We've just finished the sequencing on 200 of those patients. We also followed them at remission and at every subsequent relapse. So that's how the patient rolls through, and the clinical data follows the patient as well. So now all of our CoMMpass data is available, any scientist in the world has access to our beautiful genomic and clinical longitudinal data.

We also built the MMRF CoMMunity Gateway, where patients can go in and say, "I want to sign up and find other patients who have been through transplant," but you can also go in and sign up and say, "Hi. I have

deletion of chromosome 17p. I have translocation of chromosomes 4 and 14.” As patients move into those sites, what they will find is that we provide an expert in that category that actually cares deeply about that genomic alteration, and they will then start to discover clinical trials that are appropriate for their subtype. And that’s how we start to begin the education process that is so critically important in myeloma.

So while that personalized medicine plan is off and running and new hypotheses are coming in, we are now building the next business plan called “Drive to the Clinic.”

We have started focusing on the BRAF mutation, having found from our initial genomic paper (published in *Nature*) that four percent of myeloma patients have a BRAF mutation, which was a new finding. We then found a pharmaceutical company that had a BRAF inhibitor, which they were studying in a clinical trial. Four myeloma patients had been enrolled in two years. Of those patients, all four had had some form of a remission, and one of those patients went into a complete remission.

So now the question becomes how would we develop a model that would work for all of the companies with BRAF inhibitors? We would make sure they had access to the 4,000 samples in our tissue bank, so they could learn anything they needed about their target and their drug. Then we would give them access to the amazing principal investigators that had been doing

You are here because you are innovators in a field of personalized medicine, which I can promise you is going to change the lives not of one patient, but of thousands and thousands.

the 50 trials we have already started. And then we would work with them to pre-identify all of their patients. And then because we’re always talking to FDA, we would continue our discussions on unmet medical need and how to move things through the FDA pathway as quickly as possible. And then, of course, we would continue working on a panel that we’re working on right now, so that we can continue to screen all the genes in myeloma patients and help develop a diagnostic.

The work I did early on as a patient told me that I had a certain translocation in myeloma that made my disease the highest risk myeloma there was. It was a translocation called “4;14.” The day I found that out, because the data was published based on my twin sister and myself, I remember reading it and just driving around in my car thinking I have this horrible disease, and not only do I have this horrible disease, I have the highest risk form of this disease. And I remember thinking to myself: Why does anybody do this? Why do I want to know my genomics? And now I have to go home and look at my family and figure this out.

Knowledge is a wonderful thing, because when I later went into active

disease, my doctors knew with that translocation of 4;14 that there was a good drug for me called Velcade—and a drug called Revlimid. I was one of the first patients ever put on the combination of those drugs and then went into stem cell transplant with my identical twin, and I’ve now lived with this disease not the three years, but the 17 years that I never ever, ever expected.

So I started today by telling you that I’m just a businesswoman, and I am a mom. And I had a 1-year-old little girl. Today, she’s 19. She is a sophomore at Boston College, and she’s studying business. And I hope—I wanted her to do a science minor so badly, but I just can’t get her to do that.

And I had to tell you I also had a son, and he’s a junior in high school. And many of you that know, my whole life is about being there for them, and I accept this award on behalf of all of you because you are here because you are innovators in a field where patients desperately need you. You are here because you are innovators in a field of personalized medicine, which I can promise you is going to change the lives not of one patient, but of thousands and thousands. Today, I thank each and every one of you.



Novartis Oncology is proud to support the Personalized Medicine Coalition and congratulates this year's honoree of the Personalized Medicine Leadership Award for her remarkable achievements, dedication, and outstanding commitment to patients with multiple myeloma

Kathy Giusti,

Founder and CEO

Multiple Myeloma Research Foundation

Novartis Oncology, a business unit within the Novartis group of companies, strives to become the world's premier oncology business by discovering and developing novel therapies that may improve and extend the lives of people living with cancer and rare diseases.

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The Global Reach of Personalized Medicine

BY GEOFFREY S. GINSBURG, M.D., PH.D

Director | Genomic Medicine | Institute for Genome Sciences & Policy | Executive Director | Center for Personalized and Precision Medicine | Professor of Medicine and Pathology | Duke University School of Medicine | Professor of Biomedical Engineering | Duke Pratt School of Engineering

THIS MONTH WAS a landmark one for genomic and personalized medicine globally in efforts supported by the World Economic Forum (WEF) and the National Human Genome Research Institute (NHGRI). The WEF's Global Agenda Council (GAC) on Personalized and Precision Health (PPH) (<http://www.weforum.org/content/global-agenda-council-personalized-precision-medicine-2012-2014>) launched three major initiatives to support this dynamic field. Chaired by Victor Dzau, M.D., Chancellor of the Duke Health System, the GAC membership includes Francis Collins, M.D., Ph.D., NIH Director, Margaret Hamburg, M.D., Food and Drug Administration (FDA) Commissioner, and Jeff Drazen, M.D., New England Journal of Medicine editor as well as global leaders from Singapore, Switzerland, the United States, and India. The goals of the GAC are to advance the strategies



Geoffrey S. Ginsburg, M.D., Ph.D., is the Founding Director for Genomic Medicine in the Duke Institute for Genome Sciences & Policy.

of PPH to the advantage of all stakeholders—academic health centers, pharmaceutical companies, diagnostic firms, payers, and other broad participants in the PPH ecosystem.

The GAC met in Abu Dhabi last fall to develop the plans along three major work streams in order to present in Davos this month: 1. developing the economic case for PPH (lead: Dana Goldman, University of California, San Francisco), 2. a data-sharing strategy (co-leads: Geoff Ginsburg, Duke University, David Agus, M.D., University of Southern

California), and 3. a global harmonization of regulatory strategies globally (lead: Margaret Hamburg, FDA).

In 2014, the Global Agenda Council plans to discuss:

- A white paper on the economics in a variety of PPH scenarios—risk assessment, improved diagnostic acumen, and pharmacogenomics, where the overarching goal is to develop a framework and strategy for investment.
- A data-sharing framework that will unleash the value of siloed assets across both industry and academia—in order to accelerate the science and create value in the biomarker, diagnostics, and therapeutics landscapes.
- A landscape analysis and strategy plan to harmonize legal and regulatory opportunities in various regions of the globe—a pathway to enable personalized product development

Also this month the NHGRI, the Institute of Medicine and the Duke Center for Personalized and Precision Medicine sponsored the first Global Leaders in Genomic Medicine meeting (<http://www.genome.gov/27555775>) this past January 8–9, 2014 in Washington, D.C. The meeting, chaired by myself and co-chairs Teri Manolio, M.D., Ph.D., and Eric Green, M.D., Ph.D., from NHGRI,

gathered more than 50 leaders of institutes, centers, health care delivery systems and health ministries from more than 25 nations to highlight their genomic medicine implementation activities, enumerate the challenges, and develop a global roadmap toward creating an international community for personalized medicine. Some of the featured initiatives were the 100,000 genomes project from Genomics England, Genome Arabia, the Canadian Genomics and Personalized Health Competition, and the Implementing Genomics in Medicine (<http://www.genome.gov/27554264>) initiative in the US along with genomic medicine initiatives in Thailand, India, Estonia, Korea, Japan, Belgium, Sweden, and others.

It was particularly enlightening to see how much genomic medicine implementation activity worldwide is taking place today. However, meeting attendees, like the Personalized Medicine Coalition, acknowledged the challenges to the implementation

It was particularly enlightening to see how much genomic medicine implementation activity worldwide is taking place today. However, meeting attendees, like the Personalize Medicine Coalition, acknowledged the challenges to the implementation of genomic and personalized medicine.

of genomic and personalized medicine that formed an agenda for the group as it moves forward. These challenges include:

- Evidence of efficacy or effectiveness
- Inadequate reimbursement
- Unclear evidentiary thresholds
- Bioinformatics and electronic medical record infrastructure
- Access to point-of-care education and clinical decision support
- Expertise and training programs
- Return on investment

It was decided to create an international steering group to develop a collective agenda for facilitating genomic medicine implementation. The steering group will oversee the

activities of six international working groups (below) to develop common strategies, collaborations, and pilot projects.

These working groups include:

- Information Technology/Bioinformatics
- Education
- Evidence collection/generation
- Pharmacogenomics
- Policy
- Communications

The membership and composition of the working groups and their overall charges is still being established. In the coming weeks, more information will be available on the website.

An Agenda for Personalized Medicine

continued from page 2

now characterizes the payer-diagnostic company relationship. A second summit with BIO in Washington, D.C., that examines evidence development from the perspective of patients, providers, and payers is also planned.

Third, because the business model for developing personalized

medicine products is not well understood in either the pharmaceutical or diagnostic industries, PMC plans to examine those challenges by discussing the obstacles to development and seeking consensus around best practices and policy proposals that will encourage linking therapy to molecular diagnostics, leading to

a published paper that will inform the field.

In addition, building on the focus group-based study published last year that analyzed the public's understanding of personalized medicine, PMC has commissioned a national scientific survey to better appreciate public perceptions of the field.

Yoshihiko Hatanaka, President and Chief Executive Officer of Astellas Pharma, Inc., underscored the global reach of personalized medicine in his keynote remarks. He noted that the Astellas oncology pipeline had emphasized personalized medicine and the globalization of clinical trials had presented an opportunity to study diverse populations and identify unique subsets of patients.

To advance personalized medicine, Mr. Hatanaka called for:

- increasing funding for basic research,
- ensuring prescribing physicians have the right tools, and
- making sure patients have access to appropriate testing.

Edward Abrahams, President, PMC, and Bill Dalton Ph.D., M.D., PMC Chair, Founding Director of Moffitt's Personalized Medicine Institute, and CEO of M2Gen, presented the PMC's Ninth Annual Award for Leadership in Personalized Medicine to Kathy Giusti, Founder & CEO, Multiple Myeloma Research Foundation. The two cited Giusti's experience and leadership in introducing the principles of personalized medicine to find a cure for multiple myeloma (see page 10 for excerpts from Kathy Giusti's extraordinary acceptance speech).

A panel discussion on "The Future is Now: Genomic Sequencing at Work in the Clinic" featured personal medical experiences that had been aided by clinical genomic sequencing, but also noted the importance of demonstrating clinical utility, a precondition for third-party payment for sequencing. During the Q&A, the panelists noted that they had been fortunate to receive the diagnostic and treatment that they did because of their employment affiliation—Life Technologies in the case of Timothy Harkins and Washington University in the case of Lukas Wartman.

In her keynote, "CMS Beneficiaries and the Role of Personalized Medicine in Improving American



Yoshihiko Hatanaka, President and CEO of Astellas Pharma, Inc., calls for increased funding.

Healthcare," Shari Ling, M.D., Deputy Chief Medical Officer, Centers for Medicare and Medicaid Services (CMS), discussed some of the issues regarding patient access to new technologies in the context of CMS' broader perspective of understanding the science based on adequate evidence. She reiterated CMS policy that all tests must demonstrate ACCE criteria (analytic validity, clinical validity, clinical utility and associated ethical, legal and social implications) to earn coverage, and encouraged use of Coverage with Evidence Determination. "If there is promise for a technology but evidence is insufficient," she said, "we must focus on getting the evidence to fill gaps."

The most lively exchange of the meeting came in the Q&A period

for the panel "Building an Evidence Base to Support Molecular Diagnostic Reimbursement" between panelist Reed Tuckson, M.D., former United HealthGroup executive, and Raju Kucherlapati.

Tuckson presented a cold dose of realism when he said, "Every new thing in health care costs more than the old thing—unlike other industries." He encouraged value-based reimbursement in health care but entreated the players to work together to produce better evidence and to not "play the system."

Kucherlapati challenged the payers on the panel, saying: "The top three issues preventing the movement of personalized medicine are reimbursement, reimbursement, reimbursement." He faulted insurers for focusing on diagnostics in health care costs as a red herring, noting that they account for a tiny segment of health care costs—less than two percent. He argued, for example, that demonstrating clinical utility to justify the genome sequence of a cancer patient is virtually impossible. After nine years of meetings, Kucherlapati concluded that he had not heard a single new idea from payers about solving the issues surrounding reimbursement of PM, perhaps because they do not regard it as their responsibility.

"The top three issues preventing the movement of personalized medicine are reimbursement, reimbursement, reimbursement." — Raju Kucherlapati

PMC Announces Two New Board Members

Appointments Deepen Expertise in Drug Development, Payer Community

The Personalized Medicine Coalition (PMC) announced the appointment of Steven D. Averbuch, M.D., Vice President, Translational Clinical Development & Pharmacodiagnosics, Bristol-Myers Squibb, and Michael Kolodziej, M.D., National Medical Director, Oncology Solutions, Office of the Chief Medical Officer, Aetna, to the Coalition's board of directors for a three-year term beginning November 2013.

"With the election of these two leaders in personalized medicine—one a drug developer and the other from the payer community—PMC is well positioned to help define the future of medical progress," said Edward Abrahams, Ph.D., President, PMC.

"Over the years, the PMC has been a leader in creating awareness, facilitating stakeholder dialogue and providing roadmaps toward establishing a health care system that embraces personalized medicine," said Averbuch. "It is clear that the PMC membership is working toward a common goal to improve patient outcomes, and I am honored to be given the opportunity to contribute toward this goal as a member of the board."

In his role with Bristol-Myers Squibb, Averbuch works to optimize knowledge sharing and biomarker tools across all of R&D, and is responsible for executing the integrated



Dr. Steven D. Averbuch, left, and Dr. Michael Kolodziej have been appointed to the PMC's board of directors.

co-development of diagnostic tests as companions to the company's products. Averbuch previously held senior-level R&D positions at Merck Research Laboratories, AstraZeneca, and Mount Sinai School of Medicine. He has authored more than 60 peer-reviewed publications and book chapters, and is the co-author on one patent.

Kolodziej leads the strategic development, design and implementation of Aetna's evidence-based, patient-focused cancer care programs. Previous positions include Partner at New York Oncology, Chairman of the U.S. Oncology Pharmacy and Therapeutics Committee, and Medical Director for Oncology Services for U.S. Oncology. Kolodziej has published several manuscripts

and given many presentations on the cost of cancer care, oncology reimbursement reform, and the use of evidence-based treatment guidelines to control cost care in oncology.

"I am honored to serve on the board of the Personalized Medicine Coalition. Personalized medicine has the potential to revolutionize health care. We will enter a new era where care is truly patient-focused and patient-specific. The Personalized Medicine Coalition will be a leader in charting the course," said Kolodziej.

The appointments are in conjunction with the expiring terms of Joanne Armstrong, M.D., M.P.H., Aetna, and Nancy Simonian, M.D., Syros Pharmaceutical Inc., who rotated off the board at year-end 2013.

PMC announces the hire of Phyllis Frosst, Ph.D., as Vice President of Science Policy

Frosst comes to PMC from the National Center for Advancing Translational Sciences (NCATS), where she served as Senior Advisor for Policy, Communications and Strategic Alliances. NCATS is the newest of the National Institutes of Health (NIH) institutes. It was established to transform the translational science process so that new treatments and cures for disease can be delivered to patients faster. Frosst brings more than a decade of successful experience in translating science into actionable policy so informed decisions can be made to advance the mission of personalized medicine.

Prior to NCATS, Frosst was Head of Policy and Program Analysis at the National Human Genome Research Institute (NHGRI), NIH, where she directed policy development and served as the liaison to Capitol Hill.

In her role at PMC, Frosst will bring her expertise from within the science and policy sectors to reach consensus on policy issues impacting

personalized medicine and share those views with thought leaders and decision-makers. Frosst holds bachelor's and master's degrees with honors from McGill University in Montreal, Canada, and a doctorate in Cell and Molecular Structure and Chemistry from The Scripps Research Institute in La Jolla, California.

PMC announces Faswillla Sampson's promotion to Director of Operations and Secretary to the PMC Board of Directors.

Sampson has more than seven years of project management, managerial, and strategic planning experience in the nonprofit sector. She holds a B.A. from the University of Mary Washington and M.S. in Administration from Central Michigan University. In her new role as Director of Operations and Secretary to the Board, Sampson will oversee all aspects of the organization's financial management, human resources, information technologies and facilities in an effort to improve the efficiency of all divisions within the organization. She also serves as liaison to the PMC board.



Left, Phyllis Frosst, Ph.D., and Faswillla Sampson.

PMC'S NEWEST MEMBERS

as of Jan. 1, 2014

CBI

Centro de Genomas

Clinical Reference Laboratory, Inc.

Diaxonhit

Emdeon

Foley Hoag LLP

GeneCentric Diagnostics, Inc.

Instituto de Salud Carlos III

Millennium Laboratories

OncoPlex Diagnostics

One Disease at a Time

Ontario Genomics Institute

Opus Three LLC

ProDuCt Project Development
Consulting JSC

Proove Biosciences

Quintiles

Quorum Consulting

Sanofi

Society for Women's
Health Research (SWHR)

Sutter Health

The Quebec Network for
Personalized Health Care

Rutgers Cancer Institute
of New Jersey

The Personalized Medicine Coalition presents
the **10th Annual Luncheon Address** on

THE STATE OF PERSONALIZED MEDICINE

The annual PMC luncheon address at the National Press Club serves as a forum to discuss key issues facing personalized medicine with leaders in health care.



Featuring a keynote address by

Patrick Conway, M.D.

Chief Medical Officer

Centers for Medicare & Medicaid Services

Thursday March 13, 2014 • Noon – 2:00 p.m. ET

The National Press Club • Washington, DC

To learn more and register, visit:

www.personalizedmedicinecoalition.org



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