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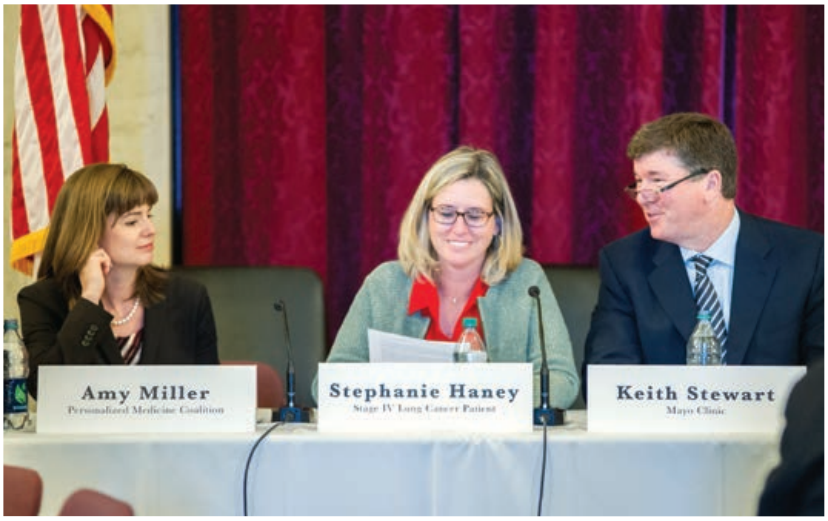
PUBLIC POLICY BRIEF
Congress Responds to Challenges in the Field

SCIENCE POLICY BRIEF
PMC Tackles Personalized Medicine Integration

FEATURE STORY
David Flockhart Reflects on a Lifetime of Achievement

Stephanie's Story



Stephanie Haney, center, describes how personalized medicine has provided eight quality years of life for her and her family at PMC's Capitol Hill briefing on July 16, 2015, alongside PMC's Amy M. Miller, Ph.D. (left) and Mayo Clinic's Keith Stewart, M.B., Ch.B.

BY EDWARD ABRAHAMS, PH.D., PMC PRESIDENT

In 2007 when Stephanie Haney, age 39, working mother of two small children in central Pennsylvania and a non-smoker, was diagnosed with stage IV lung cancer, she was told that she would be dead within one year. As she puts it, she "wasn't permitted to think" she "could 'beat' this." Her doctors did not want to offer any false hope.

Eight years later on July 16, Stephanie Haney kicked off a Senate briefing organized by PMC on the issues facing personalized medicine, including its value, cost and challenges.

Like most cancer patients, in 2007 Haney was given traditional chemotherapy, which just made her, in her words, "weak, sick and depressed." But she was also prescribed tarceva with avastin. Together, they extended

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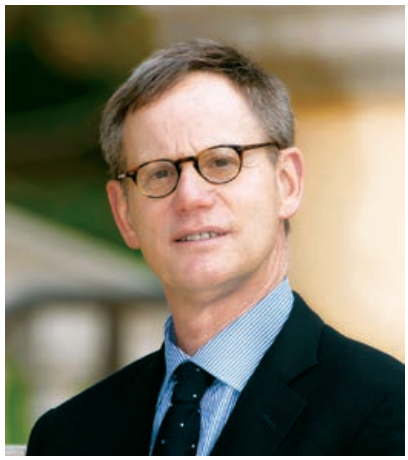
her life for more than two years, which bought her time to enroll in a new clinical trial to test if lung cancer patients with a particular genetic mutation would respond to a new drug, Xalkori. Although only four percent of lung cancer tumors have the ALK mutation, hers was one of them.

On Xalkori, which had few side effects, she was able to return to the active life she had known before her diagnosis. Unfortunately, after three years, the cancer spread to her brain. But Haney was again lucky, and discovered a second clinical trial for another ALK drug, Zykadia. Unlike Xalkori, Zykadia crosses the blood-brain barrier, and is therefore effective in patients with brain metastases. She has been taking it for about a year and half, saying, "In nearly eight years I have never achieved remission . . . [but] I have lived a virtually symptom-free, normal existence with my daughters, with hope for more."

Stephanie Haney's story is what Michael Joyner, M.D., and other skeptics regarding the promise of personalized medicine call an anecdote, or an n-of-one study, provocative perhaps but misleading and dangerous if its implications are followed. Writing on the opinion pages of *The New York Times* for January 29, Joyner, an anesthesiologist and physiologist



PMC President Edward Abrahams

at the Mayo Clinic, took issue with the premise of President Obama's Precision Medicine Initiative — "one of the greatest opportunities," in the President's words, "for new medical breakthroughs that we have ever seen." Joyner claims that personalized medicine is "unlikely to make most of us healthier." Calling it "moonshot medicine," he contends that we would be better off controlling our diets, exercising more and not smoking than understanding our genomes.

Although I doubt he would want to tell that to Stephanie Haney or her oncologists, his views are finding their way into the medical mainstream as well as the political arena, where they could wreak significant damage by discouraging investment in research and demanding impossible-to-meet standards of evidence before diagnostic and therapeutic products are approved and paid for.

Writing with a colleague, Nigel Paneth, M.D., M.P.H., an epidemiologist at Michigan State University, Joyner contends in an article published in the prestigious *Journal of the American Medical Association* on June 22 that personalized medicine has overpromised and underdelivered. And, worse, in a view that is becoming more prevalent among many stakeholders, including some oncologists, the targeted therapeutics coming out of our new discoveries of individual variation, they say, cost too much, even if they are more effective, safer and developed for smaller markets. With price tags of sometimes more than $100,000 a year, it is said, we cannot afford them, nor do they, it is also alleged, extend life by all that much.

Let's let Stephanie Haney have the last word here. She understands, as she told the attendees at PMC's briefing, "that many of these drugs have average progression-free and overall survival statistics that seem unimpressive to the public and simply give it reason to question drug costs and vilify drug companies. The incremental improvements demonstrated in the numbers also mean, however, that some patients got dramatic results, and I am one who can appreciate that," she said. "I am so grateful to the drug companies . . . because, despite the high cost of the drugs I have been prescribed, at least these companies are doing *something*."

"I am so grateful to the drug companies . . . because, despite the high cost of the drugs I have been prescribed, at least these companies are doing *something*."

— Stephanie Haney

PMC Briefing Spotlights the Field as Senate Prepares to Address Medical Innovation

BY CHRIS WELLS, PMC COMMUNICATIONS DIRECTOR

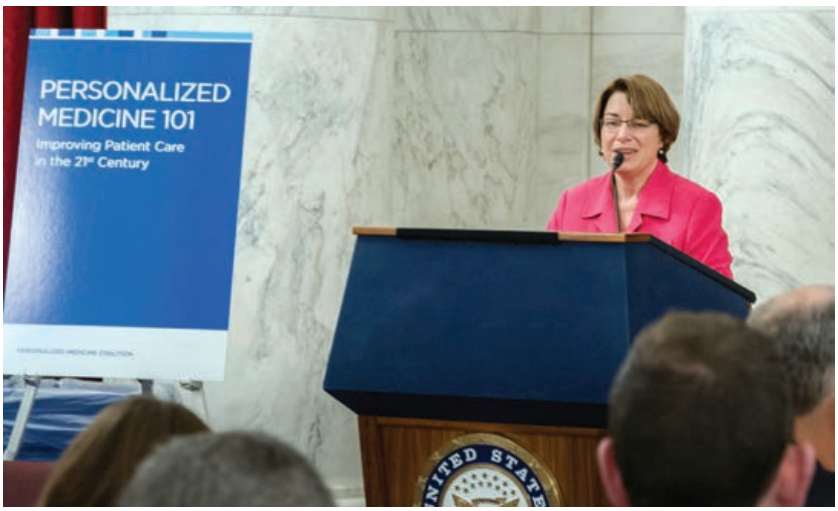
PMC'S BRIEFING ON CAPITOL HILL on July 16 emphasized the importance of personalized medicine as the Senate Health, Education, Labor and Pensions (HELP) Committee assesses its own priorities in medical innovation following the passage of the 21st Century Cures bill in the House, with Sen. Amy Klobuchar (D-MN) leading the way in advocating for funding of personalized medicine research initiatives.

"Precision medicine got a lift from the president talking about it in the State of the Union, but we need to continue on that," Klobuchar told the standing-room-only crowd of more than 125, which included more than 50 Hill staffers. "We need to increase the money we invest in research."

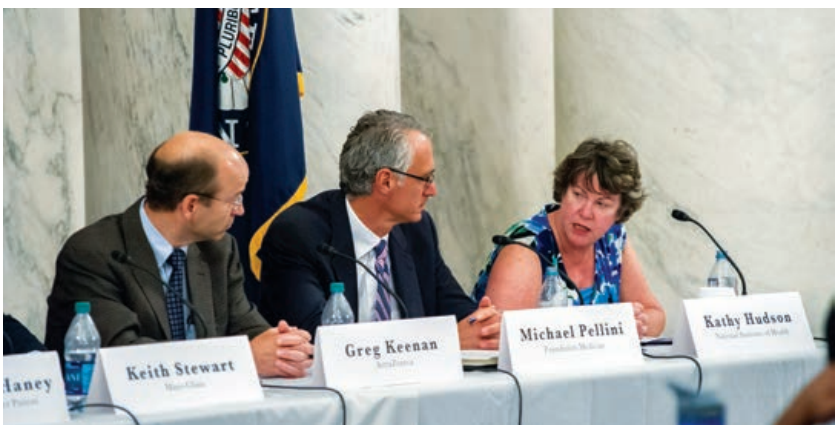
Many of the panelists echoed Sen. Klobuchar's emphasis on research funding.

Foundation Medicine CEO Michael Pellini, M.D., noted that the personalized medicine era is inevitable. But how quickly we get there, he said, depends on favorable public policies, especially in reimbursement, which he said is particularly challenging because decisions are often decentralized at the Centers for Medicare and Medicaid Services (CMS).

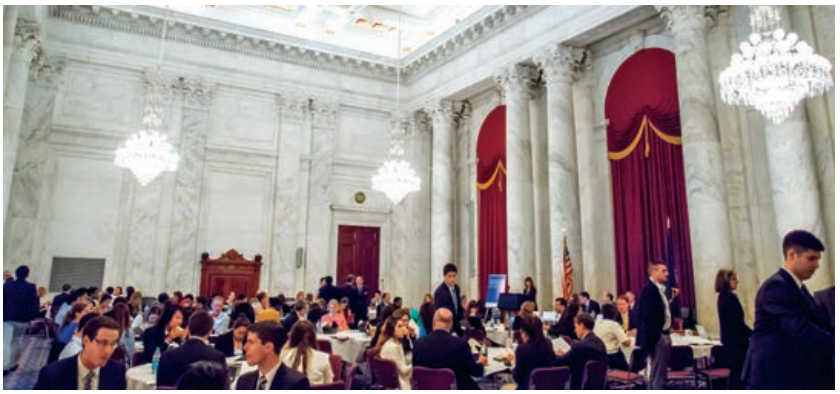
For her part, National Institutes of Health (NIH) Deputy Director for Science, Outreach and Policy Kathy Hudson, Ph.D., said the agency plans



The Honorable Amy Klobuchar (D-MN) advocates for continued support of and investment in personalized medicine.



AstraZeneca Vice President of Medical Affairs and U.S. Head Medical Officer Greg Keenan, M.D. (left), Foundation Medicine CEO Michael Pellini, M.D. (center) and National Institutes of Health Deputy Director for Science, Outreach and Policy Kathy Hudson, Ph.D., participate in a panel discussion in the Russell Senate Office Building's Kennedy Caucus Room.



The crowd gathers in preparation for Klobuchar's remarks and the panel discussion.

to begin building a volunteer cohort that will enable research to explore the molecular underpinnings of disease in the beginning of the fiscal year, as part of President Obama's Precision Medicine Initiative (PMI). She did add, however, that the initiative will have to move slower if lawmakers decide to pass a continuing resolution extending fiscal year 2015 funding levels. In that case, she said, the initiative would likely begin with funding opportunity announcements, instead of the development of the cohort.

The briefing was co-hosted by PMC and Sens. Klobuchar and Orrin Hatch (R-UT). Designed to provide an overview of what personalized medicine is and why it is important, it also featured comments from Stephanie Haney, a stage IV lung cancer patient (see cover story), Keith Stewart, M.B., Ch.B., director of Mayo Clinic's Center for Individualized Medicine, and Greg Keenan, M.D., vice president of medical affairs and U.S. head medical officer at AstraZeneca. Amy M. Miller, Ph.D., PMC executive vice president, moderated the discussion.

Personalized Medicine at the Indiana Institute

An Interview With David Flockhart

BY BARBARA LEWIS

David Flockhart, M.D., Ph.D.
credit: Zach Hetrick

David Flockhart, M.D., Ph.D., director of the Indiana Institute for Personalized Medicine at Indiana University, is a pioneer in the field. He developed the Cytochrome P450 Drug Interaction Table, which provides information on how an individual will react to and metabolize certain drugs, and used the tool to help determine his own treatment when he was diagnosed with glioblastoma multiforme, a brain cancer, in 2014. He is currently the chair of the National Institutes of Health (NIH) Implementing GeNomics In PracTicE (IGNITE) network and a member of FDA's advisory committee on clinical pharmacology.

What follows is an interview with Flockhart that was conducted in June of 2015 by Barbara Lewis, an award-winning medical journalist who hosted the public radio program "Sound Medicine" for 15 years.

Q: When did you first get interested in personalized medicine?

A: I was a medical resident at Georgetown University in 1988. I had a patient whose blood pressure wouldn't drop with what I thought was a powerful diuretic. I asked my attending physician why and he said there were a ton of possible reasons. He listed them off and it humiliated me. Maybe because it humiliated me, it also bugged me. So I started looking at the research for answers. The patient was African-American. Studies showed that diuretics don't work well in African-Americans. I wanted to understand why. I got into personalized medicine from treating individual patients.

Q: How did the Indiana Institute for Personalized Medicine (IIPM) get its start?

A: I have worked with drugs all my life. It is immensely frustrating that it takes so long to develop new drugs or to understand existing drugs. In 2000, we started the first personalized medicine institute at Indiana University School of Medicine. The idea was to put a bunch of smart people together from all different schools — medicine, nursing and informatics, all in the same building. So much of innovation, creativity and productive effectiveness has purely to do with proximity.

Q: What do you feel is your most significant contribution to personalized medicine?

A: The development of the [P450 Drug Interaction Table] that allows practicing health care professionals to anticipate and manage a large range of drug interactions that are based on the P450 system. This has allowed the

"It is immensely frustrating that it takes so long to develop new drugs or to understand existing drugs. In 2000, we started the first personalized medicine institute at Indiana University School of Medicine. The idea was to put a bunch of smart people together from all different schools — medicine, nursing and informatics, all in the same building."

individualized management of drug interactions in practice environments all over the world.

Q: What was the most transformative contribution IIPM has made to personalized medicine so far?

A: Our work on the breast cancer drug tamoxifen was truly transformative in that it demonstrated for the first time that a genetic variant could result in a decrease in the efficacy of an important drug. Numerous examples of this phenomenon now exist, but our group was the first to identify [endoxifen as an active metabolite of tamoxifen], and to isolate and name it as a new drug.

Q: Name two new developments coming out of IIPM.

A: Jamie Renbarger's work on the development of new techniques for measuring neuropathy caused by vincristine and other chemotherapeutic drugs is relevant in this context. Her work has made it possible to individualize the dosing of this important class of drugs.

Janet Carpenter has developed a wearable device that gives an accurate record of the number of hot flashes a woman has during a 24-hour period. You put it in on the skin of a woman's sternum and it measures the resistance from one side of the sternum to the other side. Before its invention, hot flashes were underreported using event monitors. The new device has led to better care and quality of life for menopausal women and those put into menopause through cancer treatment.

"We want to change the world, and medicine in particular. The means of changing medicine is to put the power — the devices and the information — into the hands of individual patients. Basically, we have to cut out the middleman."

Q: What is unique to the research you are doing in Indiana?

A: We can determine the cost and the economic impact of just one test in addition to its clinical utility. We can measure what the individual parts cost, and what the value of a test is to the health system in addition to the patient. Very few systems can tell you the cost of introducing a single test. This is being done [as part of an NIH grant program called IGNITE], which funds the Eskenazi Health program in Indianapolis, INGENIOUS. It will enroll 6,000 patients in three years.

Q: You have used personalized medicine in your own treatment for glioblastoma multiforme. What lesson have you learned as a patient?

A: The answer will surprise you. It is the simple act of caring that really matters. Of course, the advances of knowing what drugs my cancer is more likely to respond to are important. The skill of my surgeon is important, but what matters most when you are undergoing treatment is a kind word, a touch, the simple act of caring.

Q: That fits into what you see as the next frontier of personalized medicine...

A: We need to deal with "administrative toxicity," or the many ways that the administration of medicine affects the patient. For example, I have 17 prescriptions. What if I could go to the pharmacy once a month? That would be great. The pharmacist could provide a schedule of what drugs to take at what time on what day. Personalized care plans would be simple and wouldn't cost a lot. No one is paying attention to administrative toxicity. Personalized medicine could fix that.

Q: What is your vision or goal for IIPM?

A: The answer to that is simple. We want to change the world, and medicine in particular. The means of changing medicine is to put the power — the devices and the information — into the hands of individual patients. Basically, we have to cut out the middleman. That is not to say the art of constructing a differential diagnosis isn't still very important. But relative to now, there is going to be a lot more self-administered medicine. It's going on, as we speak, in the fitness business, in monitoring sleep, measuring movement, etc. It is the fastest growing new health market in the United States.

Congress Responds to Personalized Medicine Challenges

BY AMY M. MILLER, PH.D., PMC EXECUTIVE VICE PRESIDENT

The field of personalized medicine has made significant advances over the last year. For example, a PMC analysis revealed that the percentage of FDA's annual drug approvals that are personalized medicines continues to rise, with personalized medicines accounting for 20 percent of the agency's approvals in 2014 (see page 10). We see this trend continuing. In addition, a PMC-funded study confirmed that the therapeutics industry is fully committed to the field, with personalized medicines now representing more than 40 percent of therapeutics in development (see page 10). But perhaps the most significant change is the recognition among policymakers that the legal landscape must align with these new realities.

The House, for example, recently passed its *21st Century Cures* bill, which contains many provisions related to personalized medicine, including:

- Funding increases for the National Institutes of Health (NIH) for, among other initiatives, data access advances, research collaboration facilitation, and a public-private partnership to accelerate the discovery, development and delivery of innovative cures and treatments
- Authorizations for FDA to improve patient-focused drug development, qualify and use new drug development tools and advance precision medicine through modernized clinical trial designs, along with a breakthrough designation of diagnostic tests to match the one for therapeutics
- Delivery reforms such as improvements to the local coverage decision process at the Centers for Medicare and Medicaid Services (CMS)

Although broad in scope, as Rep. Michael C. Burgess, M.D., described during his State of Personalized Medicine Address at the National Press Club earlier this year (see page 11), the bill primarily appears to be an NIH reauthorization bill. The Office of Management and Budget (OMB), the federal entity that evaluates how much bills will cost the U.S. Treasury, agrees, thus putting the entire NIH budget in the bill's score even though it only increases the agency's funding by about $9 billion over five years.

For the bill to become law, however, the United States Senate must vote on a similar piece of legislation. The Senate, aware of the *Cures*' provisions, will likely draft novel legislation focusing on FDA and NIH provisions exclusively. If two different bills are drafted, they must be combined through a conference between the chambers. Both chambers must then pass the resulting bill, which would go to the President for signature.

One very controversial area of personalized medicine policy not

continued on page 12

A PMC-funded study confirmed that the therapeutics industry is fully committed to the field, with personalized medicines now representing more than 40 percent of therapeutics in development. But perhaps the most significant change is the recognition among policymakers that the legal landscape must align with these new realities.

From Promise to Patients

Outlining the Challenges and Solutions to Clinical Integration of Personalized Medicine

BY DARYL PRITCHARD, PH.D., PMC VICE PRESIDENT OF SCIENCE POLICY

FOR THE PAST 10 YEARS, the personalized medicine community has been focused on scientific discovery in genomics and molecular biology, as well as addressing regulatory oversight and reimbursement policy barriers. Many challenges in these areas still exist, but now, increasingly, there are signs that we are beginning to move beyond these issues and toward an era of health care in which personalized medicine technologies are being regularly adopted into clinical practice.

Thanks to a thriving diagnostics industry, along with a pharmaceutical industry now clearly committed to targeted therapeutic development (see page 10) and increasing annual product approvals by FDA (see page 10), more personalized medicine products are available. But perhaps the most remarkable indication involves health care delivery systems. In just the last few years, there has been a rapid acceleration in the number of health care delivery systems that have dedicated personalized medicine programs or divisions. This trend started with a few well-known academic health centers, but increasingly includes community health care systems as well.

The integration of personalized medicine into the clinic, however, raises a new set of issues. With a fundamental change in the way medicine is practiced, from population-based, trial-and-error methods to a more personalized approach through the use of molecular information about each patient, comes substantial challenges in information management, health care delivery infrastructure and culture.

Many health care delivery institutions with personalized medicine programs are beginning to develop solutions to these challenges. Much of this work is being done in isolation, and would benefit from a structured forum to share experiences and develop best practices. A major new PMC initiative focused on the integration of personalized medicine into health care has brought together more than 45 member organizations interested or engaged in health care delivery (The PMC Health Care Working Group) to discuss common challenges regarding implementing personalized medicine strategies in clinical practice.

Major challenges have been identified, including:

- Educating and building awareness amongst patients and within the health care provider community
- Developing and implementing effective infrastructure and program operations for the delivery of personalized medicine
- Managing the large amounts of data associated with personalized care so that it is comprehensive, useful and user-friendly
- Building recognition of the value of personalized medicine and providing appropriate incentives for personalized clinical decision-making
- Overcoming ethical, legal and societal issues regarding the use of individual molecular information

PMC is partnering with the Biotechnology Industry Organization (BIO) on the *PMC/BIO Solutions Summit: Integrating Personalized Medicine into Health Care* to focus all the stakeholders on identifying the solutions that each of these health care delivery institutions has been able to develop. The results of the Summit, along with the work of the PMC working group, will be used to develop best practices, which we plan to publish in a peer-reviewed journal read by health care administrators and circulated to the larger personalized medicine community.

Integrating Personalized Medicine into Health Care A PMC/BIO Solutions Summit

Renaissance Hotel · Washington, DC

COHOSTED BY: PMC · Bio Biotechnology Industry Organization

When
Wednesday,
October 14, 2015
8:00 a.m. - 5:00 p.m.

Where
Renaissance Hotel
999 Ninth St. NW
Washington, D.C.
20001

A limited number of rooms are available.

For more information, contact David Davenport at ddavenport@personalizedmedicinecoalition.org or 202-589-1770.

The 2nd PMC/BIO Solutions Summit

The Personalized Medicine Coalition (PMC) and Biotechnology Industry Organization (BIO) are convening a national summit, *Integrating Personalized Medicine into Health Care*, on October 14, 2015 at the Renaissance Hotel in Washington, D.C., to examine the integration of personalized medicine into the health care system. The summit will focus on issues related to the adoption of personalized medicine beyond the challenges of regulation and reimbursement.

At the summit, we will discuss solutions to barriers facing personalized medicine, including:

- The relationship between published evidence, clinical guidelines and the actual utilization of personalized medicine products in the clinic
- The efficiency of utilization of personalized medicine products throughout the health care ecosystem
- The widespread and adequate education regarding the use of personalized medicine products among providers, patients and payers

Who Should Attend?

Representatives from self-insured employers, pharmacy benefit managers, investors, personalized medicine diagnostic developers and manufacturers, federal policymakers and representatives from patient organizations, provider specialty societies and clinical research organizations.

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PMC Research Explores Trend Away From 'Blockbuster' Model, Toward Targeted Therapies

BY CHRIS WELLS, PMC COMMUNICATIONS DIRECTOR

42%
Percentage of compounds now in development relying on biomarker information

33%
Expected increase in personalized medicine investment over next five years

20%
Percentage of FDA's 2014 approvals that were personalized medicines

90%
Percentage of personalized medicines that were expedited by FDA in 2014

IN A 2007 ARTICLE on the promise of personalized medicine that was published in the *Harvard Business Review*, Mara G. Aspinall, formerly of Genzyme Genetics, and Richard G. Hamermesh, Ph.D., of the Harvard Business School, identified the pharmaceutical industry's reluctance to abandon the "blockbuster" drug development model, which favors the development of drugs for as broad a patient group as possible, as a barrier to the advancement of the field. Eight years later, PMC-sponsored research illustrates clear progress in overcoming this barrier, with the industry increasingly investing in targeted therapies.

According to a new study commissioned by the Coalition and conducted by the Tufts Center for the Study of Drug Development (CSDD), the industry has doubled its R & D investment in personalized medicines since 2010. Today, personalized medicines represent 42 percent of all compounds and 73 percent of oncology compounds in development. The data suggest that the industry expects to increase its investment by an additional one-third over the next five years. Clearly, the biopharmaceutical industry is now quite willing to take advantage of scientific advancements that lend themselves to the development of targeted therapies.

So what has changed since 2007?

For one, FDA has made its support of personalized medicine clear. In her online letter announcing her resignation as FDA commissioner, which was released in February of this year, Margaret Hamburg, M.D., claimed that FDA has "ushered in the era of personalized medicine."

The numbers support that rhetoric. In a blog post published in March, FDA Center for Drug Evaluation and Research (CDER) Director Janet Woodcock, M.D., points out that 90 percent of the targeted therapies approved in recent years used one or more of FDA's expedited programs, and 60 percent were approved on the basis of one main clinical trial along with supporting evidence. But perhaps most convincing is PMC's January analysis revealing that 20 percent of FDA's 2014 approvals were personalized medicines.

All of this demonstrates FDA's commitment to approving targeted therapies efficiently.

Still, the battle is far from over. Sustaining success in getting targeted

drugs to patients will require more than just one willing agency.

According to the Tufts survey, scientific discovery and regulatory and reimbursement barriers remain the top challenges facing the industry in its effort to develop personalized medicines. With industry doing its part by investing in scientific discovery, PMC Executive Vice President Amy M. Miller, Ph.D., told *GenomeWeb* last May that policymakers can do theirs by updating regulatory and payment standards.

"Companies are confounded when regulatory and payment policies do not support targeted therapies," Miller said. "The science is so hard and the benefit to patients so striking that the rest should naturally follow, in the eyes of the developers."

11th Annual State of Personalized Medicine Luncheon Address

Rep. Michael Burgess Champions Participatory Personalized Medicine Research

BY CHRIS WELLS, PMC COMMUNICATIONS DIRECTOR

MANY STAKEHOLDERS believe patient participation in research is integral to the future of personalized medicine, and at PMC's 11th Annual State of Personalized Medicine Address on May 14, 2015, House Energy and Commerce Committee member Rep. Michael C. Burgess, M.D., said he is among them.

"When I signed on to 23andMe I clicked the box that said use my information," Burgess said. "If at some point somebody as smart as Google can put together an algorithm to really have a deliverable for picking out a person who might have a problem, I'm all for that. I want that."

Burgess was the keynote speaker at the event, which took place at the National Press Club in Washington, D.C. He also discussed the *21st*

The Honorable Michael C. Burgess, M.D. (left), and Pfizer Chairman & CEO Ian Read at PMC's 11th Annual State of Personalized Medicine Luncheon Address at the National Press Club on May 14.

Century Cures bill, the need for increased funding at the National Institutes of Health (NIH) and FDA, and how mobile technologies may impact patient care. He was particularly enthusiastic about the potential for technology to reduce the hours patients spend in the doctor's office, reflecting on his experience as an obstetrician.

"To me, precision medicine can mean something as simple as [a patient being able] to take her blood pressure at home, email me the result more or less in real time, and for me to be able to then access [it] in a more timely fashion and not have to wait until something dreadful has happened," he said.

Pfizer Chairman & CEO Ian Read, who introduced Burgess, was equally optimistic about the future, highlighting the role targeted therapies play in improving patient outcomes and increasing the efficiency of the health system. He also stressed the importance of patient participation in developing these innovative treatments.

"We need dedicated support for the generation of large-scale databases of patients' data to enable a better understanding and assessment of disease risk," he said. "It requires people who agree to share data and biospecimens, so researchers can find patents that can lead to new discoveries. It requires partnership across health care stakeholders, patient organizations, providers, payers, researchers, clinicians and drug developers."

The State of Personalized Medicine Address serves as a forum for PMC members and guests to discuss key issues facing the field. Nearly 200 people attended this year's event.

Congress Responds to Personalized Medicine Challenges

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addressed in the *21st Century Cures* bill is the regulation of diagnostic tests. As PMC has previously outlined, there are now two legal paths to market for personalized medicine diagnostic tests. As many stakeholders are well aware, FDA has expressed concern that diagnostic tests are being used to make serious health decisions without having demonstrated their safety and effectiveness to FDA. The agency has released a framework for laboratory-developed test (LDT) oversight that many from the laboratory community dislike. In response, some clinical laboratories and diagnostic kit manufacturers, long on opposite sides of this divisive issue, developed an alternative proposal and are shopping it among PMC members and Congress.
While the House did not include this provision in the *Cures* bill, the Senate is considering it.

Many in the community contend that diagnostic tests are not medical devices, and thus require different regulatory rules. Furthermore, diagnostic tests undergo modifications with more frequency than traditional medical devices. Federal regulations do not yet easily accommodate those modifications. Finally, next-generation sequencing challenges the current regulatory framework guiding diagnostic tests. Most in the community contend that new authorizations might be necessary to address this challenge.

If these bills end up focusing solely on NIH funding, we must remember that personalized medicine policies may well show up again when Congress considers FDA prescription drug and medical device user fee bills. Other policies that might need some careful consideration are those on biomarker qualification and a clear path for co-developed therapeutic and diagnostic products.

Regardless, seeing health policy thought leaders so clearly focused on personalized medicine this year suggests that some of our challenges can soon be addressed.

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iConquerMS™ Begins Paving the Way For A Personalized Era in MS Research and Clinical Care

BY ROBERT MCBURNEY, PH.D., MARCIA KEAN AND KEN BUETOW, PH.D.

Robert McBurney, Ph.D., is the CEO of Accelerated Cure Project for Multiple Sclerosis. Marcia Kean is the chairman of Feinstein Kean Healthcare. Ken Buetow, Ph.D., is the director of computational science and informatics & complex adaptive systems at Arizona State University. They are, respectively, principal investigator, communications partner, and informatics and data science partner of iConquerMS™.

Substantial progress has been made in developing therapeutics for multiple sclerosis (MS) in recent years, resulting in FDA approval of 13 disease-modifying therapeutics in eight distinct mechanistic classes. That is the good news for the estimated 400,000 individuals living with the disease in the United States. The bad news is that personalized medicine has not yet come to the MS field.

When it does, we can expect more progress. Physicians and patients currently achieve the "best" treatment for each person by trial and error, based on limited outcome and co-morbidity data, patient lifestyle concerns and clinician experience. Little is known about prescribing practices in the community or whether decision-making strategies are optimal. This "dartboard strategy" contributes to unnecessarily high human and economic burdens for people living with MS and for the U.S. health care system. Moreover, none of the disease-modifying therapeutics has convincingly demonstrated efficacy for controlling progressive forms of MS, highlighting a need for improved understanding of what drives the progressive slide into severe disability in people with progressive subtypes of the disease.

Can we achieve a new, personalized era in MS research and clinical care? Advances in other disease categories point the way to an "MS Rapid Learning System," in which each person with MS would be characterized as they are diagnosed.

Can we achieve a new, personalized era in MS research and clinical care? Advances in other disease categories point the way to an "MS Rapid Learning System," in which each person with MS would be characterized as they are diagnosed.

Such an endeavor has actually begun.

iConquerMS™ (www.iConquerMS.org) is a patient-driven research initiative established in 2014 by and for those living with MS. Funded by a Phase I award from the Patient-Centered Clinical Outcomes Institute (PCORI), iConquerMS™ is implemented by a partnership among the Accelerated Cure Project for Multiple Sclerosis (www.acceleratedcure.org), Feinstein Kean Healthcare (www.fkhealth.com) and Arizona State University (www.casi.edu). Major MS advocacy organizations contribute time and capabilities for iConquerMS™ patient recruitment, network governance and network research.

iConquerMS™ collects data contributed by thousands of consented MS patients — using validated, standards-based research instruments — and then makes the data available for research projects in the PCORI national research network (called PCORnet) as well as to scientists

worldwide for research projects exploring questions of interest to patients. The iConquerMS™ data resource is also available for pharmaceutical research and development and social science studies. In addition, through Arizona State University, iConquerMS™ offers extensive capabilities for integration of its own data with external data sets.

The initial acceptance of iConquerMS™ from the MS community has been gratifyingly positive. As of June 2015, iConquerMS™ had more than 2,000 participating patients — expanding with new registrations each day — and numerous research projects undergoing the review process.

But the challenges of creating a robust evidence-generation system are formidable. The nation has not yet solved the problem of easy access to electronic health records (EHRs) for patients, and iConquerMS™ participants sometimes struggle to obtain their own records. Reaching a relatively rare disease population requires financial and human resources that can tax the capabilities of small disease advocacy organizations. Mobilizing far-flung researchers of disparate interests to collaborate in a global network presents issues of data-sharing and research oversight.

MS is not yet a poster child for personalized medicine. But with efforts such as iConquerMS™, a new era is clearly dawning.

NIH Outlines Potential PMI Implications of 2016 Appropriations Decisions at PMC Hill Briefing

CQ Roll Call published this article following PMC's briefing on Capitol Hill, during which National Institutes of Health (NIH) Director for Science, Outreach and Policy Kathy Hudson, Ph.D., said the agency's plans to begin building a volunteer cohort that will enable precision medicine research in the beginning of the fiscal year could move slower if lawmakers decide to pass a continuing resolution extending fiscal year 2015 funding levels.

"We hope that we are in a time of regular order," she said.

See page 3 for more on the briefing, which was co-hosted by Sens. Amy Klobuchar (D-MN) and Orrin Hatch (R-UT).
CQ Roll Call (July 2015)

Seeking Broader Support, House Committee Moves Dx Regulatory Proposal into Draft Legislation

Following the translation of an alternative proposal for regulating laboratory-developed tests (LDTs) by the U.S. House of Representatives Energy & Commerce Committee into draft legislation, PMC Executive Vice President Amy M. Miller, Ph.D., explained the importance of a deliberate approach to LDT oversight to *GenomeWeb*.

"People understand that LDT regulation is problematic for personalized medicine, and want a solution that will benefit innovations in the field of diagnostics but won't halt the industry," she said.

Miller noted that the speed at which the *21st Century Cures* effort moved was not conducive to the kind of detailed analysis required to nail down the specifics of a plan to regulate LDTs.
GenomeWeb (June 2015)

Exploring Precision Medicine's Value

In this interview with *Managed Healthcare Executive*, PMC Vice President of Science Policy Daryl Pritchard, Ph.D., said he believes personalized medicine is no longer about whether the paradigm can revolutionize medicine. Instead, he contended, it is about how that revolution will happen.

"The conversation is changing from concept to reality," Pritchard said. The article described the challenges PMC's Integrating Personalized Medicine into the Health Care System initiative has begun to uncover in categories such as education, operations, data management and decision-making processes.

See page 8 for more on PMC's integration effort.
Managed Healthcare Executive (May 2015)

Burgess Still Wants to Limit FDA LDT Oversight

This article, published in *The Gray Sheet*, highlights comments about laboratory-developed test (LDT) regulation that Rep. Michael C. Burgess made at PMC's 11th Annual State of Personalized Medicine Luncheon Address. Burgess said that although he was not successful in getting legislation that would prohibit FDA from regulating LDTs into the Food and Drug Administration Safety and Innovation Act of 2012, he has not changed his position or given up on the issue.

"I still think exactly as I did then," Burgess said. "But I recognize some of the political difficulties of achieving that."

For more on Burgess' comments at the luncheon, see page 11.
The Gray Sheet (May 2015)

Small Group of Labs, Dx Manufacturers Float Alternative to FDA LDT Guidance

Following the Diagnostic Test Working Group (DTWG)'s release of a 35-page alternative framework for regulating laboratory-developed tests (LDTs), PMC Executive Vice President Amy M. Miller, Ph.D., described how the proposal differs from FDA's proposed framework.

"This working group was not constrained by law, so they started with the premise that device regulation just doesn't fit diagnostics," she said. "It doesn't fit the rapid rate of change in diagnostics in terms of the modifications that must be done to diagnostic tests."
GenomeWeb (April 2015)

House Members Close to Narrowing Down 'Cures' Package

As the House of Representatives Energy and Commerce Committee worked to refine the *21st Century Cures* draft bill, PMC Executive Vice President Amy M. Miller, Ph.D., told *CQ Roll Call* that the bill would likely have a section on improved regulation for personalized medicine products, noting that personalized medicine was an important topic throughout the discussions surrounding the bill.

"The Committee has been very clear that they are supportive of regulatory research improvements [in personalized medicine]," Miller said.
CQ Roll Call (April 2015)

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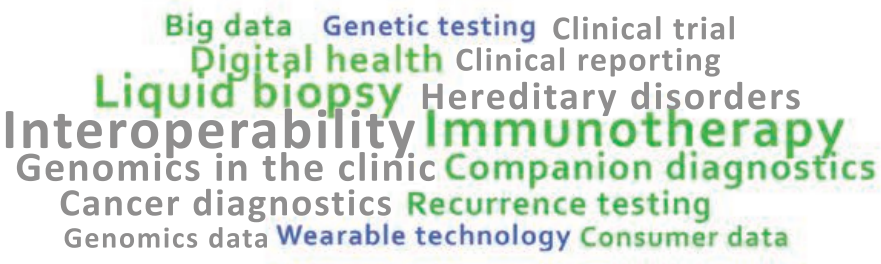

These are exciting times for personalized medicine. Affordable and faster human genome sequencing, advanced applied and translational research, and improved interoperability of health systems and databases that allows for exploration and interpretation, are converging to enable Personalized Medicine to reach the patient like never before.

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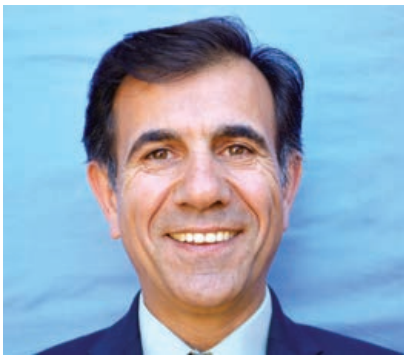
PMC Welcomes New Board Members

CONTINUING TO CONVENE leaders in personalized medicine from across the health care system, PMC has elected two representatives from both the diagnostic and pharmaceutical industries to its Board of Directors: Genomic Health Chairman of the Board, CEO and President Kim Popovits and Pfizer Executive Director and Head of Diagnostics Hakan Sakul, Ph.D.

"The additional representation from the diagnostic and pharmaceutical communities further strengthens PMC's ability to engage all of the stakeholders in health care so that they can work together to develop consensus solutions to the challenges facing the field," said PMC President Edward Abrahams, Ph.D. "The future of personalized medicine depends on collaboration among multiple sectors."

In her role at Genomic Health, Popovits leads the company's efforts to revolutionize the treatment of cancer care through genomic tests for breast, colon and prostate cancers, as well as a pipeline of liquid biopsy tests.

Kim Popovits, Chairman of the Board, CEO & President, Genomic Health



Hakan Sakul, Ph.D., Executive Director & Head of Diagnostics, Pfizer

"PMC has been at the forefront of advocating on behalf of innovators, researchers, patients and physicians, and I am delighted to join the Board to help lead the national discussion around advancing the adoption of personalized medicine through public policy," she said.

Sakul leads Pfizer's efforts in companion diagnostics across Pfizer's pharmaceutical pipeline. His work has resulted in several companion diagnostic partnerships, including the initiative that led to the simultaneous FDA approvals of Xalkori (crizotinib) and its companion diagnostic in 2011.

"As a founding member, Pfizer has been active in PMC for more than 10 years," Sakul said. "As a new Board member, I am delighted to work closer with the PMC staff and my fellow PMC Board members to help advance personalized medicine."

The appointments coincide with the expiring terms of Felix Frueh, Ph.D., Human Longevity and Jeffrey Cossman, M.D., formerly of United States Diagnostic Standards.

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www.personalizedmedicineconference.org

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