PMC PERSONALIZED MEDICINE COALITION

PERSONALIZED MEDICINE IN BRIEF

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Developments in Brief

2018

PRESIDENT'S BRIEF

Pioneering Personalized Medicine

by Edward Abrahams, Ph.D., President, PMC

To inform the Personalized Medicine Coalition's strategic plan for next year and beyond, PMC commissioned Verge Scientific Communications to conduct a survey of PMC members and non-members to find out what the most significant challenges facing the field are and how the Coalition should address them.

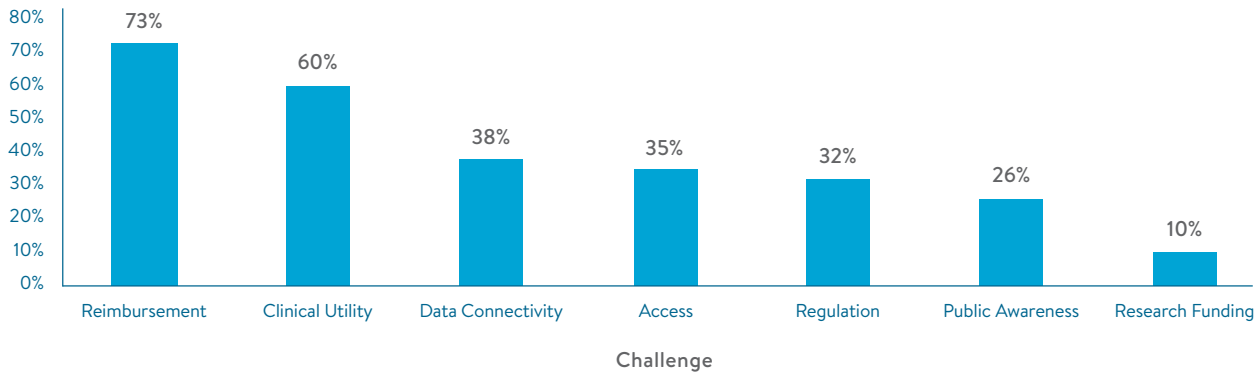
The results did not surprise us. In fact, they affirmed our present course, albeit with the caveat that we need to do a better job explaining PMC's value proposition. What follows in this newsletter is our effort to do that.

PMC's governing assumption is that paradigm shifts, especially in medicine, do not happen just because science or new technologies suggest they should. And personalized medicine is nothing less than a paradigm shift, requiring all stakeholders in health care to view the world through a new lens, one focused on individual variation and on selecting the right treatments that are targeted to only the patients who will benefit from them. The intervening variables between the science and the patient, especially regulation, reimbursement, and clinical adoption, make a crucial difference in how fast personalized medicine can and will replace one-size-fits-all/trial-and-error medicine.

Our survey found that 62 percent of those asked believe that "it is inevitable that all doctors will someday practice personalized medicine [because] the science and technology

Proponents of Personalized Medicine Cite Reimbursement, Clinical Utility as Field's Most Pressing Challenges

Based on Responses to PMC's Strategic Planning Survey (Conducted August of 2018)



Respondents to PMC's survey titled The Future of Personalized Medicine *were prompted to indicate: "Which of the following areas do you believe will present the most formidable challenges to advancing personalized medicine in the next three to five years? [Select up to three areas]"*

will demand it," whereas 38 percent thought it was not inevitable unless we "align policies and practices with the science underpinning personalized medicine." Interestingly, the answer to this question broke evenly among PMC members and non-members, suggesting that the personalized medicine community is mainly populated by optimists. On the other hand, one might conclude that 38 percent is a rather high number of people who believe that the practice of medicine is not essentially grounded in nor modified by scientific advancement.

The biggest challenges to realizing the promise of personalized medicine, our survey found, are, also not surprisingly, reimbursement and clinical utility, with data connectivity, access, regulation, public awareness, and research funding also referenced as pressing challenges that can and should be tackled through collaborative efforts such as those facilitated by PMC.

Where PMC can provide the most value, our survey found, was in influencing public policy decisions, especially reimbursement and regulation; in being "an independent voice" for the field; in advocating for access to personalized medicine products and services; and in taking a stance on specific challenges to adoption.

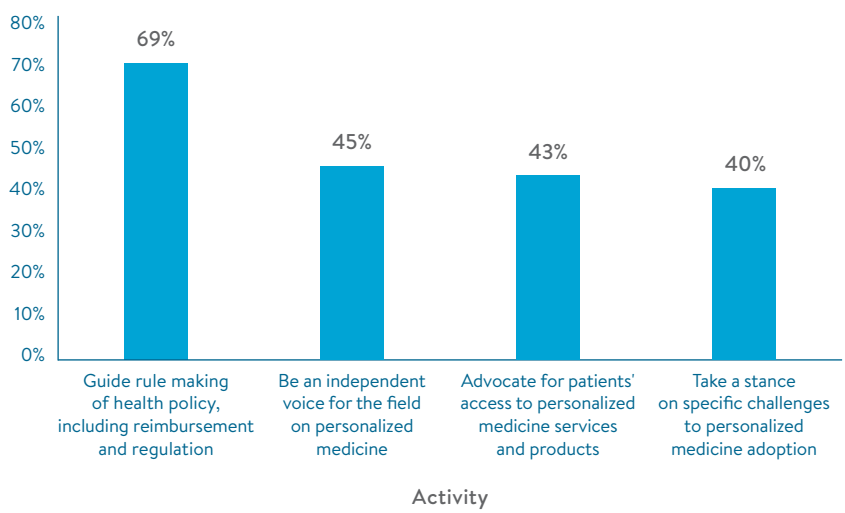
As referenced in the pages that follow, PMC is positioned to raise the profile of personalized medicine. Education is essential. As we know, too few people understand personalized medicine nor its significance, a lack of awareness that inhibits our efforts to effect the changes that are necessary to help accelerate personalized medicine's progress. PMC is also the leading advocate for creating a friendlier policy environment for investment in and adoption of personalized medicine. And, finally, we plan to continue our work, resources permitting, on building the evidence base demonstrating that personalized medicine provides better clinical outcomes and helps make the health system more efficient and less costly.

We hope that you will join other thought leaders in personalized medicine at Harvard Medical School on November 14 – 15 when, at PMC's *14th Annual Personalized Medicine Conference*, we will take on the issues facing the field in science, business, and policy.

We also hope that you will renew your membership next year or that, if you are not already a member, you will join our educational, advocacy, and evidentiary building efforts by engaging with us to bring about a better health care future based on personalized medicine.

Proponents of Personalized Medicine Believe PMC Can Provide the Most Value by Shaping Policies, Being an 'Independent Voice' for the Field

Based on Responses to PMC's Strategic Planning Survey (Conducted August of 2018)



Respondents to PMC's survey titled The Future of Personalized Medicine *were prompted to indicate: "Where can PMC provide the greatest value to your organization in shaping personalized medicine over the next three to five years? [Please specify up to three items]"*

PUBLIC POLICY BRIEF

Though Reimbursement Landscape Remains Uncertain, Some Emerging Policies May Improve Outlook for Personalized Medicine in US

by Cynthia A. Bens, Senior Vice President, Public Policy, PMC

Although it remains unclear whether the Trump Administration is willing to embrace holistic, patient-centered assessments of value that can inform sustainable approaches to reimbursement for personalized treatments that often deliver more benefits in fewer doses, U.S. policymakers have taken a series of actions this year that may incrementally improve the outlook for personalized medicine.

Congress, for example, has passed an FY 2019 spending bill that grows the amount of public funding available for scientific research about how individual variation affects disease progression and treatment response. Congress passed the bill in September, agreeing to increase the National Institutes of Health (NIH)'s budget by $2 billion in FY 2019.

Under the leadership of Administrator Seema Verma, the U.S. Centers for Medicare and Medicaid Services (CMS) has been reluctant to adopt alternative payment models that would help facilitate personalized medicine — but the agency's incremental policy changes suggest that CMS is beginning to appreciate that personalized medicine poses novel reimbursement challenges.

The bill allocates significantly more funding than the $34.2 billion request for NIH put forward by the Trump Administration. It also calls for specific investments in the *All of Us* Research Program and implementation of the *21st Century Cures Act*, both of which PMC requested in testimonies provided to Senate and House subcommittees earlier this year.

For its part, the Food and Drug Administration (FDA) has largely aligned its priorities with Congress' bipartisan interest in advancing personalized medicine.

In April, FDA finalized guidance documents on next-generation sequencing (NGS) tests that will likely help these important personalized medicine products reach the market faster. The two final guidance documents, which were released in draft in 2016, outline an innovative regulatory oversight approach for novel NGS-based tests based on the use of FDA-recognized databases to support clinical claims and ensure the accurate clinical evaluation of genomic test results. PMC encouraged FDA to further refine the pathway in a comment letter about the draft guidance documents submitted in October of 2016, in which PMC applauded the agency for its "creativity and progressive approach to policy development."

Under Commissioner Scott Gottlieb, M.D., FDA is also expanding the scope of its efforts to clarify its approach to regulating digital health software, which promise to help facilitate personalized care regimens. In June, FDA released an updated working model of its pre-certification program for these software, and Adam Berger, Ph.D., of FDA's

Personalized Medicine Staff within the Center for Devices and Radiological Health, met with PMC's members during the Coalition's Public Policy Committee meeting that same day to collect feedback on the program. The program, which FDA will continue developing and informally testing through December of 2018, creates a voluntary pathway for digital health software developers to demonstrate the excellence of their products.

Unfortunately, the Centers for Medicare and Medicaid Services (CMS) is still largely reliant on traditional payment models designed to facilitate access to daily, one-size-fits-all maintenance medications. But a few of CMS' latest policy changes suggest that the agency at least recognizes the need for updated policies that can facilitate sustainable access to personalized treatments that translate exceptionally high up-front costs into improved patient outcomes and a more cost-effective health system over the long run. In separate documents published in June titled *Medicare Hospital Inpatient Prospective Payment System Proposed Rule for FY 2019* and *Medicare National Coverage Analysis for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancer*, for example, the agency updated policies and payment rates designed to improve access to personalized CAR T-cell therapies, a set of highly personalized treatments that re-engineer a patient's own immune cells to combat certain cancers.

Expressing sentiments later echoed in the Coalition's comment letter to officials at the Department of Health and Human Services (HHS) about President Trump's *Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs*, PMC provided comment letters to CMS on both the *Proposed Rule for FY 2019* and the *National Coverage Analysis of CAR T-cell Therapy*, encouraging the agency to consider "patient-centered assessments of value" as a potential "long-term solution for adequate payment of these highly specialized medicines." The letters also encouraged the agency to increase several of the payment amounts outlined in the documents.

PMC plans to consider the policy environment for personalized medicine as part of several sessions at the *14th Annual Personalized Medicine Conference: Preparing for the New Possible* in November.

SCIENCE POLICY BRIEF

PMC Pushes Toward 'System-Wide' Integration of Personalized Medicine

Excerpted from a *Mendelspod* interview by Theral Timpson

by Theral Timpson

The following content, which was excerpted from an interview conducted by Theral Timpson, Host and Producer of Mendelspod, *a podcast devoted to the most pressing topics in biotechnology and health care, profiles PMC Senior Vice President for Science Policy Dr. Daryl Pritchard's efforts to spearhead a portfolio that will help facilitate "system-wide" integration of personalized medicine. The interview was originally published on* Mendelspod's *website on September 10, 2018. The content below has been edited for clarity.*

Theral Timpson: Welcome to *Mendelspod*.

Daryl Pritchard: Thank you Theral. Glad to be here.

TT: I want to start with a presentation you made earlier this summer at the American Society of Clinical Oncology (ASCO) Annual Meeting. You talked about a study you've done that compares multi-gene panels to single-gene tests for cancer patients. So what was the study, and what were the results?

DP: This is a question that everyone in the personalized medicine community is consistently asking: Where is the evidence demonstrating that multi-gene tests improve overall patient outcomes and are cost-effective?

We worked with health economics researchers at the Fred Hutchinson Cancer Research Center to compare data from community health care systems about patients who received multi-plex, next-generation sequencing (NGS) tests for non-small cell lung cancer (NSCLC) versus those who received standard, single-gene tests for the ALK and EGFR mutations, to see if their outcomes were better and their costs were reduced.

We saw that an additional eight percent of the NSCLC patients who received NGS tests were identified as candidates for targeted treatments, which are associated with much better outcomes. At a population level, that eight percent increase yielded significant improvements in overall outcomes for those patients.

IN THE SPOTLIGHT

Daryl Pritchard, Ph.D.

PMC Senior Vice President of Science Policy Daryl Pritchard, Ph.D., is spearheading PMC's efforts to study the clinical and economic value of personalized medicine; inform consumers about the benefits and risks associated with direct-to-consumer genetic testing; and highlight the availability of the growing number of personalized medicine products approved by the U.S. Food and Drug Administration (FDA).

TT: That sounds intuitive. If you find more biomarkers, you can send patients in more directions for targeted therapies. So what's been at the heart of the questions on this topic? Is it more money for these multi-gene tests? Is that it?

DP: Yes, they're more expensive. And payers are typically not willing to pay for a new technology until they have evidence showing that it's cost-effective. Our study showed that there is moderate cost-effectiveness associated with NGS tests.

> "What we hope to do is develop an understanding of the implementation barriers and find strategies to overcome them. We want to make sure that personalized medicine is a system-wide phenomenon, not one that is confined to key centers."
>
> Daryl Pritchard, Ph.D., PMC Senior Vice President of Science Policy

The key finding, though, is that the moderate cost-effectiveness would be much better if the test results were used more appropriately. What we found is a practice gap. A lot of the NGS testing patients received wasn't associated the way it should be with targeted treatments. So if we fix that practice gap, we will see that cost-effectiveness get even better.

TT: Say more there. What is the practice gap? Do you mean that multi-gene panels were run but patients were not always referred to an available targeted therapy?

DP: That's exactly right. Of those eight percent of patients I mentioned that were indicated through the NGS testing as potential responders to targeted therapies, only a small portion of them actually got those targeted therapies.

TT: Does that have anything to do with community providers versus a top research health care provider?

DP: There is likely an association. I am speculating when I come up with reasons as to why this practice gap occurs, but there are some things we can point to.

For one, there is an education and awareness gap. Many of the doctors may not understand how to use these results to drive their treatment decisions.

TT: OK. Let's move on to direct-to-consumer (DTC) testing. This is an area that you have done a lot of thinking about. You think this space is maturing. What are your thoughts on the world of DTC?

DP: Well, my primary thought is that what we really need to be concerned about are genetic health risk tests, which indicate your relative susceptibility to developing an illness.

At this point, I would say the U.S. Food and Drug Administration (FDA)'s strategy is that if a health care professional is part of the process for delivering those genetic test results back to consumers, the agency is not doing a strict regulatory review. But when those test results are delivered directly to a consumer without the involvement of a health care professional, it is imperative that the relevance of those genes has been scientifically validated. That's what you saw recently with 23andMe. The company cleared certain tests through FDA for "over-the-counter" use. That level of scrutiny helps alleviate concerns related to genetic health risk tests.

TT: So, you guys are called the Personalized Medicine Coalition, so let's talk about personalized medicine in general. What's standing out for you as the summer days begin to get a little shorter in 2018?

DP: Well, PMC does an analysis of FDA's new drug approvals each year. For the last four years, we have seen that an average of 25 percent of all new drug approvals are personalized medicines as we define it, meaning that there are biomarkers associated with their use listed on the FDA labels.

TT: So biomarker strategies are working out for drug companies?

DP: Absolutely.

TT: OK. That's some good, hard data.

DP: And it goes beyond that. As we see more and more gene therapies go through the development pipeline, we will really begin to be able to take advantage of our genetic knowledge for what will ultimately be very permanent treatments for patients.

TT: Are there any areas of the industry that are concerning to you guys right now?

DP: I think the concern that is starting to rise to the top is the gap between health care providers that are early adopters, which are way out ahead of the rest of the provider systems. What we hope to do is develop an understanding of the implementation barriers and find strategies to overcome them. We want to make sure that personalized medicine is a system-wide phenomenon, not one that is confined to key centers. That would lead to disparities.

TT: Daryl Pritchard, it is great talking to you. I like talking to you guys from PMC because we can go all over the place.

DP: Thank you Theral.

NEWS BRIEF

FDA Spearheads More Efficient Approval of Personalized Medicines Despite Persistent Opposition

by Christopher J. Wells, M.P.A., Vice President, Public Affairs, PMC

During his speech at the American Society of Clinical Oncology (ASCO) Annual Meeting, U.S. Food and Drug Administration (FDA) Commissioner Scott Gottlieb, M.D., wasted little time before defending his agency's evolving approach to approving cancer medicines that are designed for small patient populations.

Following a brief thank you to the event's organizers and a few words about the Administration's efforts to keep pace with new scientific developments, Gottlieb asserted that "people try to paint any change we make in our regulatory policy, or to the policy requirements we impose, as a binary choice between speed and safety." Those characterizations, he said, are "a false dichotomy."

With these remarks, Gottlieb recognized the community's emerging interest in FDA's efforts to streamline the approval of many "personalized" medicines — medicines designed specifically for patients who express biological characteristics that increase the odds that the treatment will help them. In addition to improving care for patients, personalized medicines can make the health system more efficient and effective by ensuring that therapies are prescribed to only those patients who will realize their benefits.

FDA is now approving personalized medicines based on fewer and smaller clinical trials. Gottlieb maintains that this approach allows patients with terminal illnesses and few remaining options to more easily access promising treatments that may save their lives, instead of "waiting three more years for another large, prospective, randomized trial to be completed — to confirm highly promising results already observed in an earlier clinical trial."

In oncology, for example, the cancerous cells afflicting a given patient often express genetic mutations that are not commonly present in the patient's healthy cells. Armed with this groundbreaking insight about the basic biology of the diseased cells, scientists are developing personalized

FDA Commissioner Scott Gottlieb, M.D., is overseeing the agency's efforts to streamline the approval of personalized medicines based on fewer and smaller clinical trials. Despite staunch opposition from critics who argue that the changes primarily benefit the pharmaceutical industry as opposed to patients, Gottlieb maintains that the approach is necessary to ensure that severely ill patients with few remaining options have timely access to treatments that are highly likely to benefit them.

treatments that are specifically designed to destroy only those cells that express the genetic mutations associated with cancer. Because this approach has already proven to be highly successful in patients whose cancers express a genetic mutation targeted by one of these treatments, Gottlieb argues that it is unethical to withhold experimental therapies of this kind from late-stage cancer patients who stand to benefit from them based on the presence of a genetic mutation. And because the prognosis for late-stage cancer patients is often quite poor, Gottlieb contends that these patients should be given access to personalized therapies even if the specific treatment in question has not yet been tested on a large population of patients.

Data suggest that FDA's efforts to streamline approval for personalized medicines — which began before Gottlieb's tenure as commissioner — have created incentives for industry to develop therapies that help advance personalized approaches to health care.

According to a study published in *Health Affairs* in May by a team of researchers from Harvard Medical School and the University of Queensland in Australia, personalized medicines were developed and reviewed almost two years faster than non-personalized medicines during the time period covering January 2013 through June 2017 — largely on the basis of fewer and smaller clinical trials.

And in an editorial titled "A Risky Drug Approval Lesson," *The Wall Street Journal* notes that FDA's decision in 2016 to approve Sarepta Therapeutics' Exondys 51 (eteplirsen) for a subset of patients with Duchenne muscular dystrophy, which was based on data from just 12 patients, encouraged Sarepta to develop another personalized treatment that may help even more patients with the disease.

"If FDA had cashiered [Exondys 51], Sarepta would have lacked the resources to continue its research and testing to treat Duchenne and develop what may be an even better drug," the editors wrote.

Still, some critics remain unconvinced. In a scathing critique of FDA published less than a week after Gottlieb's remarks, *The New York Times* summarized the contentions of skeptics.

The *Times*' editors argue that "it's not clear that people, as opposed to drug companies, are feeling much benefit" from streamlined approval for personalized medicines. Because FDA is now willing to approve treatments based upon smaller clinical trials and faster processes, the *Times* argues that drug companies can now "test compounds they know to be ineffective with the hope of getting a false positive result that would enable them to market a worthless medicine at an enormous profit."

PMC President Edward Abrahams, Ph.D., says responses like these exemplify the need for continued education about personalized medicine and its benefits.

In a rebuttal to the *Times*' article, Abrahams notes that "unlike FDA, which has been an engine for innovation under the direction of Scott Gottlieb and his predecessors, [the *Times*' editorial] ignores the promising implications of reforms in regulatory science that FDA has put in place to facilitate a new appreciation of how different individuals respond to selected treatments."

"By putting in place smarter policies to encourage the efficient development of personalized drugs whose safety and efficacy profiles are often higher than one-size-fits-all, trial-and-error treatments, FDA serves the interests not only of patients but also the health system, which spends too much money on ineffective treatments," Abrahams wrote.

NEWS BRIEF

Concerns Over Sales Slumps, Pricing Pushback May Hinder Investment Outlook for Personalized Medicine

by Christopher J. Wells, M.P.A., Vice President, Public Affairs, PMC

In the coming years, investors will either accelerate or inhibit progress in personalized medicine, depending on the extent to which they re-allocate capital away from the tests and treatments that facilitate one-size-fits-all medicine and toward those that facilitate new approaches to health care that target treatments to only those patients who will benefit from them.

And at the moment, not all investors are convinced that personalized medicine is good business.

The most provocative example of investors' concerns about the outlook for personalized medicine is a report published by Goldman Sachs in April titled *The Genome Revolution*, in which the global investment bank asks whether curing patients is "a sustainable business model." Recognizing that many of the most powerful personalized treatments may require just a single dose to eliminate disease in some patients, Goldman Sachs notes that these treatments have a dramatically different financial outlook than the daily maintenance medications on which the pharmaceutical industry has traditionally relied for profits.


Goldman Sachs asks in biotech research report: 'Is curing patients a sustainable business model?'

Tae Kim | @firstadopter

Published 3:15 PM ET Wed, 11 April 2018 | Updated 7:20 PM ET Wed, 11 April 2018

CNBC

In a report published in April titled *The Genome Revolution*, Goldman Sachs, a global investment bank, asks whether curing patients with personalized treatments is "a sustainable business model."

"While [the] proposition [of single-dose therapies] carries tremendous value for patients and society, it could represent a challenge for genome medicine developers looking for sustained cash flow," the report reads.

Noting that many personalized treatments promise to help patients with rare diseases, Goldman Sachs suggests in the report that biotechnology companies can mitigate cash flow challenges by focusing their efforts in personalized medicine on the most prevalent rare diseases. The report listed spinal muscular atrophy (SMA), which can be caused by mutations in at least three genes, as one such disease.

But there, too, some investors are growing skeptical.

Reacting to a first quarter sales slump for Biogen's Spinraza (nusinersen), which retains the financial benefits associated with required maintenance doses every four months and is approved by the U.S. Food and Drug Administration (FDA) for use in all patients with SMA, Geoffrey Porges, an analyst with Leerink LLC, an investment bank, said earlier this year that Biogen's plans to improve sales are unlikely to "materially alter" investors' concerns about the financial outlook for a therapy that targets a relatively small market. That sentiment raises questions about the return-on-investment companies might expect from personalized treatments that require only a single dose and treat a more limited subset of SMA patients.

"Not all investors are convinced that personalized medicine is good business."

It is important to note that sales of Spinraza have rebounded since Porges' remarks, and most onlookers recognize that isolated examples of investors' wariness do not suggest that the investment community writ large is prepared to abandon personalized medicine. The pharmaceutical industry's major players, for example, continue to invest heavily in the field through acquisitions. Novartis, for example, has purchased AveXis, a company that is also developing several personalized therapies for SMA patients.

But PMC President Edward Abrahams, Ph.D., contended in an op-ed published by *STAT News* in May that the investment outlook for personalized treatments is more fragile than the prevailing sentiment may suggest. And if ongoing concerns among policymakers and the public over the list prices of personalized treatments translate into policies that pre-empt reimbursement for personalized therapies, Abrahams says Goldman Sachs and others may be quick to direct investments away from innovative medicines that patients want and need.

To help improve the outlook for the field, the Coalition echoed Abrahams' sentiments in public letters recently submitted to the Centers for Medicare and Medicaid Services (CMS) about the agency's proposed policies related to reimbursement for chimeric antigen receptor (CAR) T-cell therapies, which are designed to permanently re-engineer a patient's own immune cells to combat leukemia in just a few doses. The Coalition also continues to advocate for the efficient regulatory approval of personalized tests and treatments and supports government funding for personalized medicine research.

"It falls on Congress, FDA and CMS to put in place an infrastructure that supports personalized medicine," Abrahams says.

The *14th Annual Personalized Medicine Conference* will examine the investment outlook for personalized medicine on November 15 during sessions titled "Considering Costs: Evaluating the Viability of Pharmaceutical and Insurance Industry Business Models in Personalized Medicine" and "Impasse or Inflection Point? — An Investment Analysis."

PMC NEWS BRIEF

PMC Adds Leaders Representing Pharmaceutical, Payer Communities to Board of Directors

by Christopher J. Wells, M.P.A., Vice President, Public Affairs, PMC

In May, PMC added PhRMA Vice President for Policy and Research Randy Burkholder and Center for Medical Technology Policy (CMTP) Founder and CEO Sean Tunis, M.D., M.Sc., to its Board of Directors, which maintains representation from all sectors of the health system.

PMC Board Chairman Stephen L. Eck, M.D., Ph.D., Chief Medical Officer, Immatics US, said the moves bolster the Coalition's capacity to identify key priorities that will advance the field.

"Randy Burkholder and Sean Tunis each have a unique perspective on the challenges facing PMC as it works to encourage the continued development of personalized tests and treatments and facilitate the adoption of those products and services in clinical settings," Eck said.

Both leaders have also demonstrated a commitment to cross-sector collaboration.

Tunis, who previously served as Chief Medical Officer at the U.S. Centers for Medicare and Medicaid Services (CMS), founded CMTP to provide a national forum for collaborative discussions on the quality and relevance of clinical research. Tunis advises a wide range of domestic and international public and private health care organizations on issues related to reimbursement and health policy.

Burkholder spearheaded PhRMA's involvement in PMC as a founding member of the Coalition, and continues to play an active role in shaping and supporting PMC's work. He also represents PhRMA in its interactions with various federal agencies and on multiple federal advisory bodies, and serves on the Steering Committee for the Partnership to Improve Patient Care.

From left to right: Randy Burkholder, Vice President, Policy and Research, PhRMA; and Sean Tunis, M.D., M.Sc., Founder, CEO, Center for Medical Technology Policy

"Burkholder and Tunis are thoughtful leaders in personalized medicine who are well-positioned to help guide PMC's work in education, advocacy and evidence development," said PMC President Edward Abrahams, Ph.D.

Burkholder and Tunis will join the Board alongside Amy Abernethy, M.D., Ph.D., Chief Medical Officer, Chief Scientific Officer, Senior Vice President, Oncology, Flatiron Health, who was recently re-elected to serve an additional term.

"Randy Burkholder and Sean Tunis each have a unique perspective on the challenges facing PMC as it works to encourage the continued development of personalized tests and treatments."

PMC Board Chairman Stephen L. Eck, M.D., Ph.D

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The 14th Annual Personalized Medicine Conference: Preparing for the New Possible will convene the world's leading researchers, investors, industry executives, policy experts, payers, clinicians, and patient advocates to define the landscape and outlook for personalized medicine in science, business, and policy. Participants will examine the infrastructure and business strategies necessary to overcome scientific obstacles, optimize public policies, and change embedded medical norms as we seek to accelerate investment in and adoption of personalized medicine.

14TH ANNUAL
PERSONALIZED MEDICINE CONFERENCE

MEDIA BRIEF

From the PMC News Desk

CMS' Decision to Allow the Use of Step Therapy for Part B Drugs in Medicare Advantage May Undermine Personalized Medicine

The U.S. Centers for Medicare and Medicaid Services (CMS) recently issued a reversal of a long-standing policy, with the revised policy allowing Medicare Advantage plans to require treatment of patients with less expensive medicines before they are eligible for more expensive options — even if a patient's doctor believes a more expensive treatment is the right one for them. The decision to allow step therapy for Part B drugs, which is intended to contain costs, does not adequately protect the physician-patient relationship and threatens to undermine the delivery of personalized medicine.

In a comment letter to the U.S. Department of Health and Human Services (HHS), PMC noted that although the Coalition "recognizes the increasing pressure to contain overall health care costs," this policy would have a negative impact on patient care.

"Requiring a patient to fail first on a less expensive treatment limits a provider's ability to prescribe a treatment based on the best available evidence and impacts a patient's ability to control his/her disease," PMC wrote. "We fear that this may lead to poorer outcomes that may not be reversible."

See *NPR*: "New Medicare Advantage Tool to Control Drug Prices Could Narrow Choices" (September 2018)

Op-Ed Belittling Personalized Medicine's Potential in Oncology Prompts PMC Response

An opinion article published earlier this month by Liz Szabo of *Kaiser Health News (KHN)* has sparked debate over personalized medicine's long-term potential.

In a pointed critique of the field's reported successes in oncology, Szabo contends that positive characterizations of personalized medicine "mislead the public." Most reports, she notes, rely on "best-case scenarios" to tout the promise of the field, without acknowledging that personalized cancer care still fails to help most cancer patients.

After the article was re-run by *The New York Times* under the headline "Are We Being Misled About Precision Medicine?," PMC President Edward Abrahams, Ph.D., submitted a letter to the editor of *KHN* arguing that although Szabo is "correct to note that precision medicine is not yet 'addressing the needs of the majority of cancer patients,' she overlooks the considerable progress in the last two decades."

In partnership with its members, the Coalition plans to develop a proposal for a comprehensive online library of information about personalized medicine and its benefits next year.

See *New York Times*: "Are We Being Misled About Precision Medicine?" (September 2018)

PMC Op-ed: 'A Focus on Cost Instead of Value Threatens the Future of Personalized Medicine'

In an op-ed published by *STAT News* on May 23 titled "A Focus on Cost Instead of Value Threatens the Future of Personalized Medicine," PMC President Edward Abrahams, Ph.D., contends that ongoing efforts to reduce list prices for drugs may unintentionally discourage the development of personalized treatments.

Abrahams notes in the op-ed that unlike one-size-fits-all, daily maintenance medications, personalized treatments are often designed to improve patients' symptoms for many years with just a few doses. He also explains that these treatments can help make the health system more efficient by targeting therapies to only those patients who will benefit from them, sparing expenses and side effects for those who will not.

See *STAT News*: "A Focus on Cost Instead of Value Threatens the Future of Personalized Medicine" (May 2018)

Survey Shows Most Americans Are Not Familiar With Personalized Medicine — But Are Excited About Field's Potential When They Learn About It

A representative survey of 1,001 Americans published in May by PMC and *GenomeWeb* shows that although most Americans are not very familiar with personalized medicine, an overwhelming majority agree that the field can provide "major" benefits to the health system.

According to *Public Perspectives on Personalized Medicine: A Survey of U.S. Public Opinion*, which PMC released at the National Press Club on May 23, 67 percent of Americans have never even heard the terms "personalized medicine" or "precision medicine," which are often used interchangeably to describe the field. Only 13 percent of respondents indicated that they feel "very informed" about the topic.

When personalized medicine was defined for them, however, 67 percent of Americans indicated a "mostly positive" reaction to it. Majorities of survey respondents indicated that the field could provide "major" benefits to the health system, and 82 percent said they want to learn more about it.

See *GenomeWeb*: "Public Awareness of Personalized Medicine Not Growing in Step With Industry, Survey Shows" (May 2018)

FDA Streamlines Path to Market for Key Personalized Medicine Tests With Final Guidances on Oversight of Next-Generation Sequencing

In April, the U.S. Food and Drug Administration (FDA) streamlined the path to market for next-generation sequencing (NGS) tests, which help facilitate personalized care regimens by allowing doctors to use a single test to assess a patient's candidacy for multiple treatments.

FDA published two final guidance documents related to the oversight of NGS tests on April 12, outlining a path to market that eliminates the need for NGS test developers to submit clinical trial data demonstrating the clinical significance of biomarkers that are already validated by FDA-recognized data sets. NGS test developers are quick to note that the expensive process of generating data about already validated biomarkers can discourage investment in NGS tests.

PMC encouraged FDA to refine and finalize the streamlined pathway for NGS tests in a comment letter about earlier drafts of the guidance documents in October of 2016.

"NGS tests help patients get on effective treatment regimens sooner," PMC President Edward Abrahams, Ph.D., explained in an article about the final guidance documents published in *Bloomberg BNA*. "With these guidance documents, FDA has created a regulatory pathway that will help encourage the integration of NGS tests into clinical care."

See *Bloomberg BNA*: "FDA Finalizes Oversight Plan for Advanced Genetic Tests" (April 2018)

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