

PERSONALIZED MEDICINE IN BRIEF

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Developments in Brief

2019

FEBRUARY 15

The U.S. Centers for Medicare and Medicaid Services (CMS) proposes coverage of an important group of personalized treatments, called chimeric antigen receptor (CAR) T-cell therapies, “with evidence development.”

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FEBRUARY 13

PMC’s *Personalized Medicine at FDA: A Progress & Outlook Report* underlines the extraordinary pace of scientific progress in personalized medicine by demonstrating that personalized medicines topped 40 percent of all new drug approvals in 2018.

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JANUARY 17

PMC President Edward Abrahams and National Alliance for Hispanic Health President & CEO Jane L. Delgado, Ph.D., publish an opinion article in *STAT News* advocating for more diverse clinical trials that can advance personalized medicine.

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JANUARY 3

Bristol-Myers Squibb caps a flurry of industry moves that bolstered the scientific prospects for personalized medicine by announcing that it will acquire Celgene for \$74 billion.

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2018

DECEMBER 22

The U.S. Congress’ failure to pass key spending bills triggers a 35-day partial government shutdown. The shutdown leaves investors wary of allocating capital toward personalized medicine tests and treatments that depend on a thriving FDA to reach the market efficiently.

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OCTOBER 25

The Trump administration proposes to tie payment rates for all treatments in the U.S. to the rates established by other countries without considering the unique value proposition associated with an incoming wave of personalized tests and treatments.

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Personalized Medicine: Creating an Ecosystem of Shared Value

by Edward Abrahams, Ph.D., PMC President



Since the Personalized Medicine Coalition was launched at the end of 2004, we have had to answer two questions. First, what is personalized medicine? And, second, why should my institution join a collective effort to change the current medical paradigm from one-size-fits-all to prescribing, in the language of personalized medicine, the right treatment to the right patient at the right time?

Over time, as personalized medicine has evolved from promise to reality, notably in treating particular kinds of cancer and certain rare diseases, the concept, at least among the readers of this publication, has become familiar. It does not require a great deal of explanation as to why and how health care that stresses — as Hippocrates put it more than two thousand decades ago — “treating the patient who has a disease rather than the disease who has the patient” makes sense. Although multiple stakeholders can and do disagree on the levels of evidence necessary to move toward personalized medicine, almost all believe that it is a good idea, especially if they are patients or want to prevent some illness.

But the second question — why should my institution join the Personalized Medicine Coalition? — is tougher and less obvious since PMC, a multi-stakeholder group that cuts across sometimes competing business models, does not fit easily into the constellation of trade associations that, more often than not, seek to prevent change.

Not infrequently do we hear people tell us that they admire our education and advocacy work but that their institution or company is not ready to commit even a small amount of resources to change the medical paradigm; that is, to influence, as it were, the space between the science and the patient so that the path from research to the bedside becomes easier. Believing in a “field of dreams,” these institutions assume that if they build it, change will come.

Recently, for example, one high-level executive in a company that had raised almost a billion dollars from investors to

develop an innovative and disruptive diagnostic technology told us that she planned to join “coalitions” following “the completion of their research.” To me, that sounds a little like Napoleon going to Moscow and worrying about what he will do after he gets there.

I discussed this challenge with past PMC chair, Bill Dalton, who is the Founder and Executive Chairman of M2Gen, a health informatics company that has created an exchange network across multiple academic health centers to study patients throughout their lifetimes. Dealing with the “what’s in it for me” issue in the academic medicine community, not known especially for its spirit of cooperation, is something not unknown to Bill. He referred me to an essay by Mark Kramer and Marc Pfitzer, “The Ecosystem of Shared Value,” published in the *Harvard Business Review* issue for January 2017.

Kramer and Pfitzer, business consultants who want to develop business opportunities *and* solve social problems, contend that “companies that turn to collective impact will not only advance social progress but also find economic opportunities that their competitors miss.” They argue that companies can create what they call “shared value” by “reconceiving products and markets, redefining productivity in the value chain, and strengthening local clusters.”

We can see this happening in personalized medicine, where academic researchers, pharmaceutical manufacturers, diagnostic companies, payers, and providers have come together, albeit willy-nilly and haltingly, and not without a lot of external and internal opposition, to create opportunities and new markets to solve health care challenges. Some estimate that the personalized medicine market now is over \$40 billion. This market, though small in comparison with the overall size of the health care market, did not exist a little over a decade ago.

But Kramer and Pfitzer also point out that if innovative initiatives like personalized medicine are to succeed, they require changing how the system works. And that, we

“If innovative initiatives like personalized medicine are to succeed, they require changing how the system works. And that requires a common agenda and especially a dedicated backbone of support from an independent and trusted organization.”

know, is easier said than done. It requires, they write, a common agenda and especially a dedicated backbone of support from an independent and trusted organization that can guide vision and strategy, build public support, and mobilize resources to ensure that all the components of the integrated system are “aligned and informed.” Among the goals of the trusted agent are to ensure that “the health of the whole system benefits each party.”

According to Dalton, this is the role PMC can and should play. In his view, the Coalition should become the “honest broker” that, in order to create collective impact,

develops solutions without preference for any particular party — with the exception of the patient.

With these thoughts in mind, I encourage you to reread PMC’s strategic plan for 2019, conveniently published on PMC’s website, noting how it is divided into three sections: education, advocacy, and evidence development. Our overarching goal, notably PMC’s effort to develop the evidence that personalized medicine works, is to become the agent of change that increases public and private investment in a new paradigm that promotes individual health and a sounder health system.



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PERSONALIZED MEDICINE CONFERENCE

The 15th Annual Personalized Medicine Conference at Harvard Medical School 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.

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Policy Developments Raise New Concerns for Personalized Medicine as FDA Sharpens Focus on Field



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

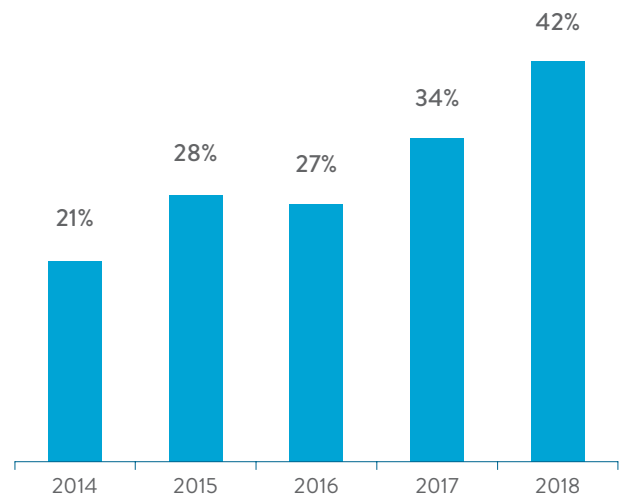
On the heels of scientific and regulatory developments that brought record numbers of personalized tests and treatments with unprecedented clinical benefits to the market last year, U.S. lawmakers have generated new concerns about the field's future in 2019 — by instigating a temporary partial shutdown of the Food and Drug Administration (FDA) that has shaken investors' confidence in the prospects for biomedical innovation in the U.S. and by embracing cost-cutting measures that may chill investment in personalized treatments that promise to translate higher up-front costs into a more effective and efficient health system.

PMC's recently released *Personalized Medicine at FDA: A Progress and Outlook Report* demonstrates that more than one of every three drugs FDA approved in 2018 is a personalized medicine, meaning that its label contains information designed to ensure that the drug is prescribed only to patients whose bodies express specific biological characteristics that make them highly likely to benefit from the treatment. The agency facilitated these results through a coordinated series of guidance documents and policy updates. In December, for example, PMC noted in its comment letter about the agency's thoughtful draft guidance document titled *Long-Term Follow-Up After Administration of Human Gene Therapy Products* that the guidance proposes modernized regulatory approaches "suitable for an era in which personalized therapies can treat disease in just a few doses by permanently changing the genes in patients' own cells."

FDA's stalwart commitment to the field has not wavered in 2019.

The agency has already advanced its effort to streamline the approval of various personalized medicine diagnostic products with three documents that outline a regulatory pathway for digital technologies that can enable real-time adjustments to personalized prevention and treatment plans. It has also hired former PMC Board Member and

Personalized Medicines Top 30% of FDA Approvals for Second Year in a Row



PMC's recently published *Personalized Medicine at FDA: A Progress and Outlook Report* documents an accelerating trend toward the development of personalized treatments. These treatments accounted for 42 percent of all new drug approvals in 2018.

personalized medicine pioneer Amy Abernethy, M.D., Ph.D., who previously served as Chief Medical Officer, Chief Scientific Officer, and Senior Vice President for Oncology at Flatiron Health, to help lead the agency as Principal Deputy Commissioner of Food and Drugs.

But Congress' failure to pass 2019 appropriations prompted a partial government shutdown between December 22, 2018, and January 28, 2019, slowing the agency's progress

“U.S. lawmakers have generated new concerns about the field’s future in 2019 — by instigating a temporary partial shutdown of the Food and Drug Administration (FDA) that has shaken investors’ confidence in the prospects for biomedical innovation in the U.S. and by embracing cost-cutting measures that may chill investment in personalized treatments that promise to translate higher up-front costs into a more effective and efficient health system.”

and concerning proponents of personalized medicine that the agency’s reduced funding would pre-empt its ability to establish a new Office of Drug Evaluation Science (ODES), issue additional policy guidance on the development of gene therapies, and advise Congressional lawmakers who are working to establish a new oversight framework for the regulation of diagnostic tests, all of which have the potential to advance personalized medicine considerably.

As PMC and more than 40 other organizations contended in a letter developed by Friends of Cancer Research and sent to President Donald Trump and key Congressional leaders in January, the shutdown “[put] the current health and safety of Americans at risk” and “put future scientific discovery and innovation in jeopardy.”

Although the shutdown has since ended, the ongoing political uncertainty and Congress’ reliance on temporary continuing resolutions to keep the government running have left investors wary of a U.S. biomedical sector that depends on FDA as a gatekeeper for the health care products that come to market.

Meanwhile, an emerging set of cost-cutting proposals from Congress and the Trump administration threatens to disrupt patient access to personalized treatments.

Congressional lawmakers and the Trump administration are seizing on an area of rare bipartisan interest to work toward alignment on a slew of sweeping policies that are designed to decrease the amounts that the U.S. government pays for broad groups of tests and treatments, without consideration of their value to patients and society. These emerging policies include proposals to decrease reimbursement rates for some diagnostic tests through the *2019 Medicare Clinical Laboratory Fee Schedule*; pay for the least expensive (and sometimes less appropriate) therapies first; and tie reimbursement rates for certain treatments to the rates set by other developed countries.

Expressing sentiments echoed in other comment letters to administration officials on each of these topics, PMC warns in its letter to the Centers for Medicare and Medicaid Services (CMS) about the agency’s proposed *International Pricing Index Model* that “continued proposals imposing blunt payment cuts or rigid clinical and cost-effectiveness standards would create significant barriers to the development of and access to innovative drugs and diagnostics.” PMC has advocated instead for the development of more sophisticated “patient-centered assessments of value” that can quantify the benefits of unprecedented personalized treatments.

In his public communications and speeches, FDA Commissioner Scott Gottlieb, M.D., continues to demonstrate that he is concerned about policy developments that are challenging his own efforts to accelerate progress in the field. Gottlieb underlined the importance of an updated regulatory framework for diagnostic tests, for example, in a blog posted on December 6.

And on the topic of reimbursement, he shares the community’s concerns.

With remarks during October’s *Future of Health Summit* that exemplify how personalized medicine challenges health systems still accustomed to facilitating access to one-size-fits-all, daily maintenance medications, Gottlieb said he is “extremely worried” that unresolved questions related to reimbursement for “potentially curative” personalized therapies that deliver long-term benefits in just a few doses may discourage the development of similar products.

“I’m extremely worried that if we don’t adapt the approach to reimbursement soon, we may foreclose the therapeutic opportunities,” Gottlieb said.

PMC will explore the policy landscape for personalized medicine during the *15th Annual Personalized Medicine Conference at Harvard Medical School* in November.

Landscape for Clinical Adoption of Personalized Medicine Still Coming into Focus, But Early Insights Underline Importance of Value Recognition



by Daryl Pritchard, Ph.D., PMC Senior Vice President, Science Policy

In a post published in January on PMC’s blog, *Education & Advocacy*, Brad Power, a patient diagnosed with lymphoma in July of 2018, reflects on his care at a well-known academic medical center that has a reputation for leading the way in the delivery of personalized medicine. He attended the 14th Annual Personalized Medicine Conference at Harvard Medical School, and noted in his blog that he did not get the personalized care that was being described by leaders at the conference, some of whom were from the same pioneering health care center where he received care. Somehow there was a disconnect. Power submits that if his experience is representative — and his encounters with other patients suggest that it is — then the pace of clinical adoption, despite the best intentions of pioneering health care providers, is still “excruciatingly slow.”

Research and development of personalized medicine technologies is stronger than ever. PMC reports in *Personalized Medicine at FDA: A Progress & Outlook Report* that more than one of every three drugs the U.S. Food and Drug Administration (FDA) approved over the last two years is a personalized medicine. Yet despite this scientific progress, it is becoming clear that personalized medicine is not a “field of dreams.” The fact that you built it does not mean that they will come. Power’s blog reminds us that unless physicians are regularly ordering personalized medicine tests and using the results to guide targeted treatment decisions as appropriate, we will be unable to translate scientific progress into improved care for patients.

PMC’s Health Care Working Group, consisting of over 40 health care delivery and clinical support organizations, helped publish “Strategies for Integrating Personalized

Medicine into Healthcare” in the journal *Personalized Medicine*, in which we identified the key challenges to the clinical adoption of personalized medicine. The challenges fall into five categories that will need to be addressed in order to effectively implement personalized medicine strategies and fully realize their potential for patients and the health system: (1) education & awareness; (2) patient empowerment; (3) value recognition; (4) infrastructure & information management; and (5) reformed health care delivery practices.

To be sure, building awareness and educating health care providers is where we need to start. There are clear case examples of a relatively small group of well-informed, advanced institutions that have implemented nationally recognized personalized medicine programs. It is not clear, however, what impact the move toward personalized medicine within these pioneering institutions has had so far on the health care system in general. In order to capture a holistic picture of the clinical adoption of personalized medicine strategies and technologies within the U.S. health care system, PMC has commissioned a more representative national survey of health care delivery organizations. This, in turn, will inform efforts to address the most critical outstanding awareness and education needs.

Implementing personalized medicine will also involve a more empowered patient. While policies that appropriately ensure privacy and security of individual health data are evolving, it is important to involve patients more holistically in their own health care decision-making. PMC Board Member and Section 32 Managing Partner Michael Pellini, M.D., predicted at the *Annual Personalized*

“The key lynchpin to clinical adoption of personalized medicine is the recognition of its value by all health care stakeholders.”

Medicine Conference that patients will soon drive personalized medicine into clinical settings with or without the assistance of health care professionals. The surge of interest in direct-to-consumer (DTC) genetic testing suggests that he is right. More than 30 million people have ordered consumer genetic tests despite uncertainty regarding the clinical impact of providing genetic information in this way and the risks associated with misinterpretation and misuse of the data. To help patients responsibly navigate the DTC testing landscape and subsequent clinical encounters, PMC is working with clinicians, industry representatives and patients to develop *A Guide to Consumer Genetic Health Testing*.

The key lynchpin to clinical adoption of personalized medicine, however, is the recognition of its value by all health care stakeholders. To this end, many within our community are working to build the evidence that can demonstrate the clinical and economic value of personalized medicine strategies. PMC commissioned a research study, for example, that examined the clinical and cost effectiveness of next-generation sequencing (NGS) for patients with non-small cell lung cancer (NSCLC). The research, which analyzed de-identified electronic medical records from a cohort of 5,688 NSCLC patients, showed that more than 35 percent of patients who had actionable mutations as determined by NGS-based diagnostic testing wound up receiving standard chemotherapy rather than more effective targeted treatments indicated by their tumor genetic

profile. The reason for this “practice gap” is not clear. The researchers found that although NGS testing has moderate cost-effectiveness in this environment, closing this practice gap would improve the cost-effectiveness of these tests considerably. Payers and providers need to understand this value proposition to facilitate improved access to personalized medicine technologies.

Addressing these education, patient empowerment and value recognition challenges will greatly accelerate the development of the health care delivery infrastructure and health information management systems of the future — systems that are built for the delivery of personalized medicine. But reforming health care delivery processes in a way that moves away from traditional fee-for-service practices and focuses on value in care at the patient level will require collaborative contributions of time and resources. Indeed, it is not as simple as the “build it, and they will come” mantra of a field of dreams. On the contrary, it will be necessary to demonstrate that the destination is worth it. PMC Board Member and Medical Director of the Moffitt Cancer Center DeBartolo Family Personalized Medicine Institute Howard McLeod, Pharm.D., who helps guide PMC’s clinical integration efforts as the Co-Chairman of its Science Policy Committee, may have said it best.

“It’s one thing to talk about personalized medicine,” McLeod explains. “It’s another to have your health system invest in it.”



“It is one thing to talk about personalized medicine,” PMC Board Member and Medical Director of the Moffitt Cancer Center DeBartolo Family Personalized Medicine Institute Howard McLeod, Pharm.D., said during the *13th Annual Personalized Medicine Conference at Harvard Medical School*. “It’s another to have your health system invest in it.” McLeod helps guide PMC’s clinical integration efforts as the Co-Chairman of its Science Policy Committee.

Amid Criticisms of Field, Proponents for Personalized Medicine Emphasize Unchanging Biological Principles of Individual Variation



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

As the *MIT Technology Review* noted late last year in an article published as part of a special “precision medicine issue,” it is currently “in vogue to question why precision medicine has not delivered more.”

Finding traction for their arguments in mainstream publications including *The New York Times* (see “Are We Being Misled About Precision Medicine?”) as well as influential smaller outlets including the *Boston Globe*-affiliated *STAT News* (see “Precision Medicine’s Rosy Predictions Haven’t Come True. We Need Fewer Promises and More Debate”), the field’s critics advance a simple formulation in which proponents of personalized medicine have grossly exaggerated the potential of an approach that is not living up to its promise. Because genetically guided therapies have not improved population health, they contend, medicine should move away from personalized medicine in favor of health care tailored to what works for the “average” patient.

As PMC noted in its response to one of these critiques, titled “Are We Being Misled About Precision Medicine?” (published in *Kaiser Health News* before being picked up by *The New York Times*), this narrative threatens to undermine public support for an evolving approach to health care that acknowledges inconvenient but immutable biological variations among individuals. The scope of this educational challenge became evident last year, when PMC commissioned a survey in partnership with *GenomeWeb* demonstrating that more than two-thirds of Americans have never even heard of “personalized” or “precision” medicine.

Actually, as PMC continues to underline through channels that include a series of articles published as part



PMC was among those who weighed in with a letter to the editor about a recent article titled “Are We Being Misled About Precision Medicine,” which was published in *Kaiser Health News* and later picked up by *The New York Times*. By “suggesting that positive characterizations of precision medicine’s potential ‘mislead the public,’” the letter warns, these kinds of articles “may undermine patients’ confidence in treatment approaches that save lives and inhibit future investment upon which improvement in cancer care depends.”

of educational inserts in *USA Today*, the available evidence suggests that the health system should be moving toward personalized medicine, not away from it.

The field’s opponents largely agree with its champions, for example, that personalized medicine based on genomic

“Unlike opponents of personalized medicine, who are content to condemn the field at a relatively early stage in its development, Mukherjee ... maintains that instead of returning to an era in which all cancer patients are treated the same way, researchers must pursue ‘a world of information beyond mutations.’”

principles has delivered clinical value to some patients, a fact that has become too visible to ignore.

To cite but one of the field’s achievements to date, personalized therapies targeting genes that are believed to fuel the spread of cancerous cells have raised the five-year survival rate for patients with non-small cell lung cancer to a level that is three times higher than the rate for patients with small cell lung cancer, which has genetic underpinnings that are still largely unknown.

But the logic of the field’s critics and champions diverges when it comes to the significance of these incremental steps forward.

Noting that the genetically guided interventions that comprise most of the personalized medicines today are only addressing the needs of relatively small subsets of patients with rare genetic mutations, skeptics contend that personalized treatment approaches, which are, by virtue of their increased sophistication, sometimes costlier, should be abandoned in favor of cheaper interventions for large groups of patients, even if only some of those patients will benefit. Investments in any other approach, they suggest, will never be worth it.

But personalized medicine’s champions acknowledge that early successes like those observed in non-small cell lung cancer are evidence that personalized medicine has begun to provide the tools necessary to combat the root causes of diseases that are influenced by a unique set of interactive molecular variables at work in each patient. Researchers will be unable to refine medicine and cure diseases, they contend, until scientists embrace research underpinning personalized medicine and decision-makers address the economic

challenges to delivering it. And although they recognize that the genetically informed approach is not a panacea for all ailments, proponents maintain that it will play an important role in a future for medicine in which interventions are tailored based on many kinds of biomarkers.

In a formulation that may prove prophetic in oncology and elsewhere, Siddhartha Mukherjee, M.D., D.Phil., summarizes this perspective in an article published in *The New York Times Magazine* in June of 2018.

He acknowledges in the article that the disappointments of some genetic studies have “fueled public criticisms of precision medicine.”

But unlike opponents of personalized medicine, who are content to condemn the field at a relatively early stage in its development, Mukherjee, anticipating the impact of more expansive metabolic analyses as well as artificial intelligence, maintains that instead of returning to an era in which all cancer patients are treated the same way, researchers must pursue “a world of information beyond mutations,” including data about the patient’s immune system as well as the unique set of “metabolic inputs that a cell needs to integrate in order to grow.”

In short, Mukherjee recognizes, as Tango Therapeutics President and CEO Barbara Weber, M.D., has also stated, that the question is not whether we “should do this.”

“We have to do this,” Weber explains. “We don’t get to decide what the biology of these diseases are, we just have to work with it.”

To meet the field’s educational needs in 2019, PMC’s strategic plan outlines a variety of initiatives designed to “raise the profile and scope of the field.”

NEWS BRIEF

Emerging Gene Therapies Push Insurers Toward Innovative Payment Models Suitable for Era of Personalized Medicine

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

Since the 1970s when researchers first documented the inheritable nature of retinitis pigmentosa, doctors have historically had the misfortune of explaining to every family impacted by the genetic eye disease that the affected patient's decreased ability to see at night and with peripheral vision is irreversible.

But Spark Therapeutics is writing a new history for this disease.

Spark won approval in December of 2017 from the U.S. Food and Drug Administration (FDA) to begin marketing its personalized treatment, Luxturna (voretigene neparvovec), which can restore eyesight for some patients with retinitis pigmentosa by correcting a harmful genetic

mutation in the RPE65 gene that causes the disease. In so doing, the company has developed a "gene therapy" that brings new hope to patients with a devastating illness.

According to a database curated by Informa Pharma Intelligence, biopharmaceutical companies are developing more than 700 gene therapies, each of which is designed to treat or even cure disease by altering the ways in which a patient's cells are functioning.

But like the two other FDA-approved treatments that re-engineer certain biological processes to combat a disease, Luxturna, which carries a list price of \$425,000 per eye treated, is challenging payers to find innovative ways to finance a new chapter in medicine that is characterized



By delivering longer lasting benefits through fewer treatments, gene therapies like Spark Therapeutics' Luxturna (voretigene neparvovec), pictured here, present a unique value proposition to patients and society.

“[Gene therapies] are challenging payers to find innovative ways to finance a new chapter in medicine that is characterized by therapies that translate higher up-front costs into unprecedented downstream benefits.”

by therapies that translate higher up-front costs into unprecedented downstream benefits. Although Spark has agreed to rebate the price of the therapy if it does not work as intended, the company is facing significant reimbursement challenges as it seeks to market Luxturna. Investors caution that if payers and biopharmaceutical industry leaders are unable to agree on payment models that allow companies to recoup the costs associated with these kinds of treatments — without inhibiting patient access — Wall Street may redirect its capital away from personalized medicine.

The conundrum remains vexing, but solutions have begun to emerge.

Novartis CEO Vas Narasimhan, M.D., for example, has suggested that reinsurance companies, which earn premiums from insurance companies by agreeing to absorb some of the costs associated with unlikely but devastating events that may impact the insurer’s clients (e.g., natural disasters), may have a role to play in helping the health system absorb the costs associated with gene therapies like Novartis’ AVXS-101 for spinal muscular dystrophy, which the company believes may be worth up to \$4 million per treatment.

There are reasons to believe reinsurance is viable.

The *Financial Times* notes in an article published in December of last year that big reinsurance companies like Swiss Re and Munich Re are on the hunt for alternative revenue sources, especially because they are facing increased competition from rival sources of risk capital. Reinsurers already work with employer health insurance plans, and they have begun to expand into the medical sector. Reinsurers teamed up with the World Bank in 2017, for example, to serve as a backdrop against future Ebola

pandemics. Narasimhan speculates that reinsurers might play a similar role in guarding against “the catastrophic case of a child having one of these conditions.”

Pioneering leaders in the health insurance and biotechnology industries have also indicated a willingness to spearhead new payment models through which insurers pay for gene therapies in installments.

Bluebird Bio CEO Nick Leschly, for example, said last month that the company is exploring installment plans for its forthcoming gene therapy for a rare inherited blood disease. Leschly says the treatment will be priced below the \$2.1 million that Bluebird estimates the therapy is worth, in the interest of patient access. According to Bluebird’s favored terms, insurance companies would pay as little as 20 percent of the total cost for the therapy each year for five years — but only for as long as the therapy works as intended.

“We only get paid if we do what we said we’d do,” Leschly explains.

PMC Board Member and Harvard Pilgrim Health Care Chief Medical Officer and Senior Vice President Michael Sherman, M.D., M.B.A., M.S., who has already spearheaded similar “outcomes-based” contracts with biopharmaceutical companies, said these contracts are “the best model I know of when you have high-cost treatments of unknown durability.”

Overall, insurance company representatives are unsure of what the future holds — but they almost all agree that it looks different than the past.

“With gene therapy, you’re going to have drugs routinely priced at over \$1 million, and it’s really going to push all of us to think in a different way,” said Steve Miller, M.D., Chief Clinical Officer, Cigna.

Personalized Medicine: Promise or Potential?

Remarks during the 14th Annual Personalized Medicine
Conference at Harvard Medical School

by David P. King, J.D., Chairman, CEO, LabCorp



The following remarks were prepared by LabCorp Chairman and CEO David P. King, J.D., for his keynote address at the 14th Annual Personalized Medicine Conference at Harvard Medical School on November 14. Except when indicated by brackets, they are presented below exactly as they were prepared.

Thank you, Paul, for that generous introduction.

Thanks to Ed Abrahams and the Personalized Medicine Coalition for inviting me to speak today at this impressive gathering. It's an honor to be here with so many thought leaders in health care and in personalized medicine.

It has been said that personalized medicine offers the promise of a future in which we can predict, prevent, and treat disease at the individual patient level in exciting new ways. It has also been said that the promise of precision medicine will lead to better clinical outcomes at a reduced cost.

But the dictionary tells us that a "promise" is "a declaration or assurance that one will do a particular thing or that a particular thing will happen." I don't think personalized medicine is a promise, I think it is a field with some notable early successes and great potential. And like anything with potential, there are many opportunities and many obstacles to navigate. I'd like to spend some time talking about them today.

I've taken a bit of liberty with my topic today — I will go beyond the clinical laboratory industry and discuss:

- How is the role of diagnostics evolving within the world of personalized medicine?
- What are the challenges that we face, and do those challenges suggest that we are going to miss opportunities to advance personalized medicine?
- What do we need to do to move forward and make the promise of personalized medicine come to life?

We can find a paradigm for the opportunities and challenges of personalized medicine in a recent story from *Kaiser Health News* entitled "Pricey Precision Medicine Often Financially Toxic For Cancer Patients," about Kristen

Kilmer, a 41-year-old woman from Spearfish, South Dakota. Ms. Kilmer was diagnosed three years ago with incurable breast cancer caused by a mutation in the PALB2 gene. The mutation was discovered by a next-generation sequencing test, which may cost up to \$6,000 and is often not covered by commercial insurance.

Ms. Kilmer has been successfully treated for three years with Lynparza[®], which is an FDA-approved therapy — but only for breast cancer patients with a BRCA mutation. Her insurer declines to cover the drug, calling it experimental. The insurer says it makes coverage decisions based on "published, randomized data about the safety and efficacy of the requested drugs." Ms. Kilmer is being treated with Lynparza because she searched for experimental treatments, drives 12 hours round-trip to participate in a clinical trial, and has spent much of the past three years "battling insurance officials and begging drug companies for financial assistance." The manufacturer recently decided to stop providing her the drug without charge and the out-of-pocket cost to Ms. Kilmer would be \$17,000 per month — on top of the approximately \$81,000 her family has already spent out-of-pocket treating her cancer. She decided to discontinue treatment because she did not want to burden her family with the cost. "Within hours" of the article running in *KHN* and *USA Today*, the manufacturer called to inform Ms. Kilmer that it would continue to provide financial aid to support her taking the drug.

Really, I could just stop here because this story so perfectly frames the issues and opportunities of personalized medicine, but Ed would probably not be happy to have 35 minutes to spend up here making shadow bunnies. So, let's examine this in a bit more detail.

The story starts with a diagnosis of breast cancer and a sequencing test to try to identify the mutation. Here Ms. Kilmer was fortunate: someone involved in her treatment knew to order this kind of test. This should not be assumed: one major obstacle to personalized medicine and targeted

therapies is lack of physician awareness of companion diagnostics and therapy options. Broadly speaking, there is still insufficient emphasis in physician education on the use of lab testing to inform drug therapy, whether in companion diagnostics, pharmacogenomics or therapeutic drug monitoring. Indeed, although over 95 percent of clinicians said in a survey that they know genetics affects drug response, only a small percentage said they have used genetics to aid drug therapy in practice. The number one challenge they cited is needing guidance to translate genetic results into clinical actions.

Physicians need better access to clinical decision support and access to genetic counselors who can help them choose the right test, interpret the results and explain them to the patient. Here we encounter a second major obstacle to personalized medicine: the restrictive view of payers about genetic counseling. Again, doctors say they don't know what tests to order or how to understand them; genetic counselors are degreed professionals trained to help with these issues; yet payers don't pay appropriately for genetic counseling services (if they pay at all) and, to my puzzlement, won't allow LabCorp genetic counselors to assist physicians because they say we have a "conflict" in that our genetic counselors would try to generate more orders for genetic testing.

I submit that part of the problem here is a lack of understanding about diagnostics. A diagnostic is not just a test that one decides whether to cover and pay for. A diagnostic is a complex system of reagents, instruments, software, algorithms, procedures, interpretations and support services, all of which must be included — and done correctly — to make the final product valid and clinically meaningful. Genetic counselors are part of the diagnostic service we offer; their code of ethics specifically forbids exploiting clients for personal or institutional advantage; and I can tell you from personal experience that they would never encourage ordering tests that would not benefit their patient.

So physician awareness and underutilization of the skills of genetic counselors are obstacles. As I said, however, Ms. Kilmer was fortunate: she had the laboratory test. In doing so, she took advantage of next-generation sequencing (NGS), one of the major opportunities for personalized medicine. NGS is absolutely at the forefront of innovation; it has revealed the complexity and commonality of molecular alterations in various cancers, allowing the development of testing panels for frequent and actionable variants. NGS has also improved the accuracy and limits of detection for finding somatic mutations and can interrogate hundreds of genes for various alterations, including single nucleotide variants, small insertions or deletions, copy number variants and translocations. This innovation translates into enormous potential for non-invasive prenatal testing (NIPT) using the mother's blood to detect fetal chromosomal abnormalities, as well as potentially accelerating the

detection of cancer and monitoring its progression through cell-free DNA circulating in the bloodstream rather than through invasive biopsies with variable accuracy.

Yet the opportunity of NGS runs into the third major obstacle to personalized medicine: payer coverage. A spokesperson for America's Health Insurance Plans (AHIP), a trade association for managed care plans, commented in the *KHN* story that recent scientific advances in genetic testing and genome mapping are "remarkable and noteworthy," but that AHIP needs a more definitive answer to how genetic testing truly ties to informing care and improving health outcomes. Interestingly, in March, Medicare announced it would cover NGS for certain advanced cancers when the test is an FDA-approved or -cleared companion diagnostic with an FDA-approved or -cleared indication for use in that patient's cancer, or a Medicare administrative contractor determines that coverage is appropriate.

By contrast, consistent with the AHIP response, commercial payers have been mostly unresponsive to paying for genetic testing, particularly when performed by NGS. In our experience, payment for genetic testing in breast cancer patients is largely limited to BRCA 1 and 2 mutations. Adding genes beyond these traditional ones sharply reduces coverage. Ms. Kilmer's mutation is in PALB2, a protein that binds to and co-localizes with the BRCA2 early onset protein, and may function in tumor suppression. It is not, however, part of the standard BRCA 1/2 test.

I did some research on the South Dakota State Employees' Health Plan and discovered that there are six criteria for covering genetic testing. The fourth criterion is: "The testing method is scientifically proven to be valid in detecting the specified gene and the relationship between the gene and treatment have been validated through randomized control trials and presented in peer-reviewed scientific literature demonstrating health outcomes will be improved." Given this, I venture to say that Ms. Kilmer's test was not covered and that few genetic tests would be.

[Demonstrating that the relationship between the gene and treatment has been validated through randomized controlled trials is problematic in the first instance; randomized trials are designed to prove the safety and efficacy of drugs and devices, not the validity and utility of diagnostics. Given the small number of patients involved in most personalized medicine applications, a randomized trial — even if meaningful — would be enormously expensive to conduct and take years to conclude. Imagine the difficulty in then securing publication of the results in "peer-reviewed scientific literature demonstrating health outcomes will be improved." In my view, the coverage criteria are heavily stacked against diagnostics — and therefore against patients as well.]¹

The focus in the coverage decision on how genetic testing improves health outcomes (demonstrated through the

1. Bracketed remarks were not delivered verbatim. They are included here for clarity.



LabCorp Chairman and CEO David P. King, J.D., delivered the remarks republished here at Harvard Medical School on November 14, during his keynote address at the *14th Annual Personalized Medicine Conference: Preparing for the New Possible*.

just-discussed requirement of randomized controlled trials published in peer-reviewed scientific literature) is really a way of asking the question: what is value? Of course, a genetic test alone is not going to improve health outcomes; the potential of personalized medicine is that the insights from the test and the actions taken as a result will do so. So the value question begins with a false premise that invariably leads to the misleading answer: “no, the test does not improve health outcomes.”

But let’s assume there could be a meaningful inquiry about the test result alone. If a companion diagnostic indicates that the patient will respond to a drug that is FDA-approved for the indication, does that improve health outcomes? If the test indicates that a patient will not respond to the drug — does that improve health outcomes? What would be covered? And how will a test that is intended to identify a small group of responders meet Ms. Kilmer’s insurance company’s requirement of validation through randomized control trials that will be nigh impossible to conduct, much less to secure peer-reviewed publications?

And there is more. What set of patients should we look at to determine value in the precision medicine calculation? We know how to measure quality-adjusted life years saved, but these assessments are typically made across a population, not at the individual level. How should such concepts apply to Ms. Kilmer or people like her when the idea of precision medicine is individualized treatment for a particular disease? Can we meaningfully characterize patient groups for which personalized medicine treatment will be cost-effective given the genetic heterogeneity of the U.S. population? How do we think about value when a drug that is indicated by genetic testing as one the patient will respond to can be thwarted by intra-tumor heterogeneity? This concern is particularly acute given the “hope or hype” debate about personalized medicine and policy questions

about whether society can realize more value from addressing social determinants of health such as diet, smoking cessation and weight loss than from personalized medicine.

Parenthetically, innovative NIPT faces similar obstacles; payers limit coverage because they don’t want to incur the cost of testing for the average-risk population. Yet we know that the incidence of microdeletions in the average-risk population (those women not considered high-risk) is significant, that they are detectable by NIPT, that NGS will continue to refine our understanding of those risks, and that NIPT is the safest and cheapest way to do this. The difference in viewpoints about the scope of coverage for NIPT is essentially a difference in perspective on how we define value of diagnostics and value of personalized medicine. Unless we come to some agreement among stakeholders about how to solve these admittedly difficult questions of assessing value, we will have a difficult time realizing the full potential of personalized medicine.

Ms. Kilmer’s personal perseverance highlights another important opportunity for personalized medicine: the growing role of the consumer. The increasing use of non-disease-oriented, consumer-initiated testing will lead to better patient understanding of disease-oriented diagnostics and a reduction of the information asymmetry between patient and doctor. I support this trend, I applaud it and I think it is one of the most important things that is going to happen in personalized medicine in the next five to ten years. The prevalence of patient support and advocacy groups, opinions from “Dr. Google” and social media generally will democratize the understanding of disease treatment and progression.

Yet this opportunity also presents critical challenges to the credibility of personalized medicine. Having taken a few of these consumer-initiated tests myself, I can say unequivocally that the results are usually ... equivocal.

I recently took a test to determine my fitness level and the report I received stated in consecutive paragraphs: “People like you are more likely to have greater endurance for long distance sports,” followed by “You’re more likely to have a harder time excelling at endurance sports. This is due to a genetic disadvantage in endurance and muscle efficiency caused by lower blood flow.” But wait ... two paragraphs later: “People like you may have greater blood flow to your muscles and therefore more strength, which is especially beneficial for exercises that require power over endurance.” What to do? Quit exercising? Quit taking over-the-counter tests?

This test is by no means alone in the market. One of my colleagues has tried essentially every in-home test available to consumers — some of which are clinically oriented, some of which are not. Having reviewed the results, I can say without equivocation that analytical precision, reproducibility and concordance with gold standard reference lab testing is lacking in many consumer offerings. Scientific evidence for testing such as “food sensitivity,” “sleep and stress,” and “egg count”

is scant, if it exists at all. And yet, as we think about personalized medicine and clinical trials, the validity of the data is fundamental. And as we move further away from the gold standard with wearables and in-home testing, I don't in any way want to suggest that these are not important and valuable trends, but we need to be aware that they have brought into question the credibility of our field. Yet the consumer, the provider and the health care system all assume that lab data, however obtained, is both clinically valid and accurately measured. The lessons from a blood testing unicorn of recent memory do not seem to have been fully absorbed.

I will say: precision, reliability and reproducibility of data are absolutely critical if we are to improve the delivery of care (whether through personalized medicine, clinical trials, value-based care or simply the routine encounter in the physician office), and in my judgment, the assumption that the results from any test ordered online are accurate is not a well-founded assumption. The FDA has said: "A bad test is every bit as bad as a bad drug." Truer words were never spoken.

Finally, let's talk about the manufacturer's reaction to the publication of the story. This too is fairly typical of personalized medicine: the plight of a gravely ill patient is highlighted in the media and the "bad guy" caves in and pays. I'm not saying by any means that Ms. Kilmer should have been denied the drug — indeed, the article suggests that it is critical to her survival. I am saying that this type of decision-making process undercuts our ability to realize the full potential of personalized medicine. And it is particularly acute in oncology, where the media regularly publish stories about decisions by insurers or drug companies to refuse to pay for therapy. In many cases there is no clinical evidence supporting that therapy and in some, diagnostic testing has even determined that the patient will not respond, but the narrative typically includes emotional vignettes and a quote from the treating physician that the patient "has run out of options and needs a chance."

This is not the promise of personalized medicine. The promise of personalized medicine begins with administering the right drug to the right patient, but it does not end there. That is a short-term win but not the long-term vision. To illustrate my point, let's move away from personalized medicine applications for oncology because the critically ill patient with no other treatment option clouds the picture there.

Think about a disease that causes much misery and decreased productivity, like migraines. Suppose there is a drug for migraines that is accompanied by a companion diagnostic. With consistent use of the companion diagnostic by providers and reimbursement by payers, we can: (1) get the effective drug to the patients who will respond to it and relieve their suffering, (2) identify the non-responders and use other treatments, and (3) use the non-responder patient set as the basis for research on and development of other therapies. Thus, personalized medicine creates a

virtuous cycle: optimal patient care for responders, prompt exploration of alternatives for non-responders with cost savings from not using a drug that won't work, and robust discovery pipelines for new drugs. This model can be repeated for any disease state that is subject to exploration through genetic and biomarker discovery — although it will work best for those in which the stakeholders are not faced with a decision to "give the patient a drug or let them die."

To summarize the personalized medicine equation: we have the challenges of physician understanding, accurate interpretation of test results, coverage and the determination of value, and confidence in the results. On the other side of the equation, we have the opportunities of technology such as NGS, better utilization of genetic counselors, expanding knowledge bases and the ability to broaden the application to new disease states. Here then is my "short list" of things we need to do to make the potential of precision medicine a reality:

- Close the educational gaps for the key constituents and stakeholders:
 - Consumers
 - Providers
 - Payers
 - Other thought leaders
- Convene a cross-disciplinary group of interested parties to agree on the value equation:
 - How do we define the value of personalized medicine and how do we determine it in individual cases?
 - How do we balance the potential of short-term cost increases due to deploying expensive drugs versus long-term savings from avoiding ineffective therapies, providing better treatment and improving outcomes?
 - How do we assess the value of personalized medicine in the hierarchy of other health care initiatives?
- Through appropriate diagnostic-specific policy balancing innovation and access with patient protection, get serious about requiring:
 - Scientific basis for tests
 - Clinical relevance to the question we are trying to answer
 - Reliability and reproducibility
- Expand the case for personalized medicine beyond oncology and create the virtuous cycle of effective treatment for responders, alternative treatment for non-responders and exploration of new treatments.

From my perspective, it is an exciting time for personalized medicine. We have made great progress, yet in my judgment we are still closer to the beginning of the journey than to the end. The opportunity ahead is enormous and I hope that we will find the will, the alignment of interests, and the focus needed to complete the journey and keep the promise.

PMC Board Bolsters Coalition's Capacity by Finalizing New Slate of Board Officers, Establishing Public, Science Policy Committee Chair Positions

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

In a series of moves designed to enhance PMC's capacity to anticipate and tackle key regulatory, reimbursement and clinical adoption issues through education, advocacy and evidence development, the Coalition's Board of Directors has finalized its slate of 2019 officers and established chair positions for PMC's Public and Science Policy Committees.

Stephen L. Eck, M.D., Ph.D., who is spearheading the efforts of Immatics U.S. to develop highly personalized immunotherapies for cancer patients as the company's Chief Medical Officer, will continue to serve as PMC Board Chairman. The remainder of the officers include several diagnostics industry representatives in Peter Maag, Ph.D., CEO, President, CareDx (Board Treasurer), Kimberly Popovits, Chairman of the Board, CEO, Genomic Health (Board Secretary), and Jay G. Wohlgenuth, M.D., Chief Medical Officer, Senior Vice President, Quest Diagnostics (Board Vice Chair).

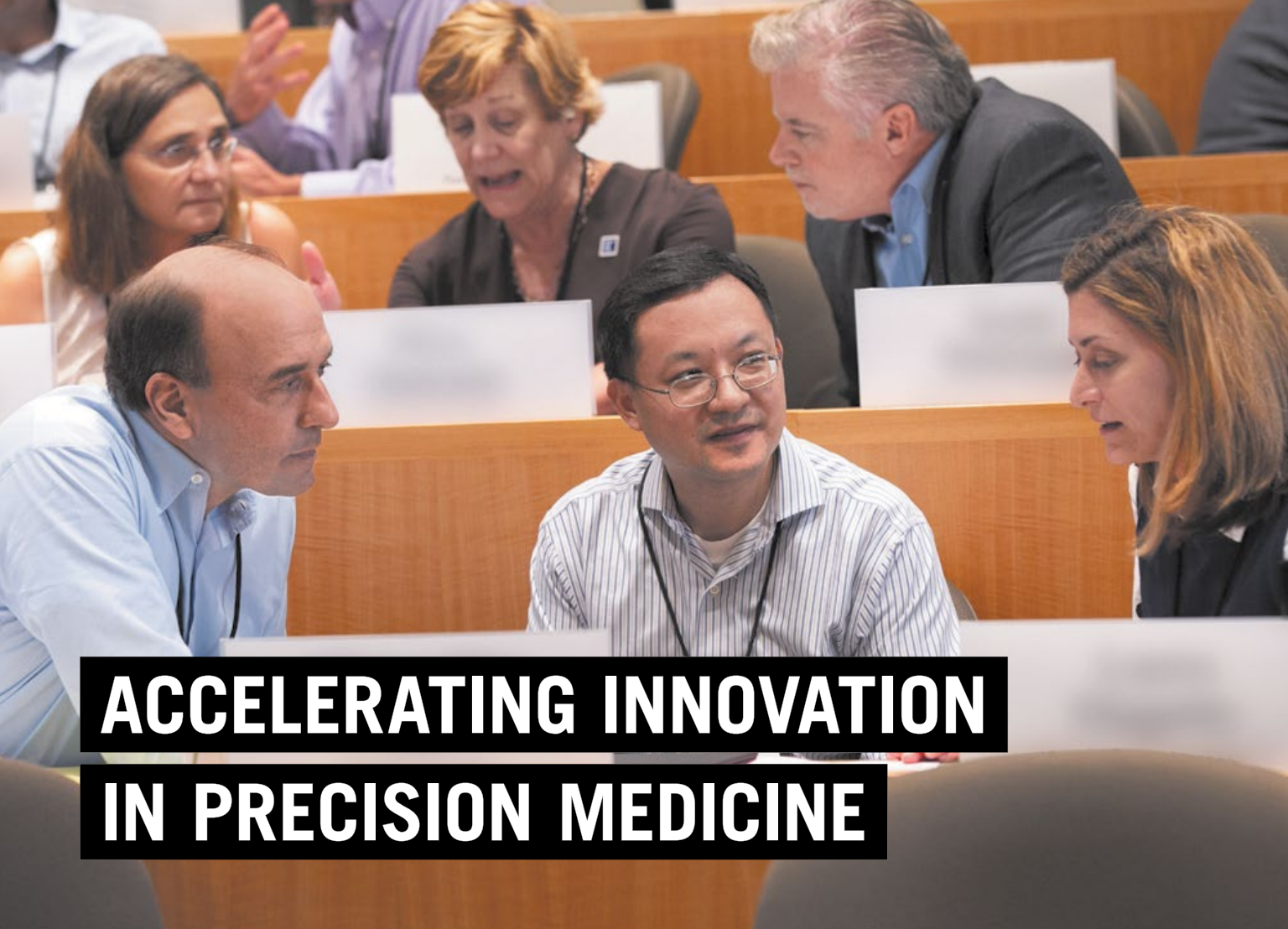
To ensure that PMC is exceptionally well-positioned along the critical fronts of public policy and clinical adoption, the officers, in collaboration with their 15 Board colleagues representing patients, clinicians, and the diagnostics, IT, pharmaceutical, and health insurance industries, selected Michael Sherman, M.D., M.B.A., M.S., Chief

Medical Officer, Senior Vice President, Harvard Pilgrim Health Care, and Paul Williams, Director, Federal Affairs and Policy, Bristol-Myers Squibb, to co-chair the Public Policy Committee; and Howard McLeod, Pharm.D., Medical Director, DeBartolo Family Personalized Medicine Institute, Moffitt Cancer Center, and Lincoln Nadauld, M.D., Executive Director, Precision Medicine, Precision Genomics, Intermountain Healthcare, to co-chair the Science Policy Committee.

M2Gen Founder and Executive Chairman William S. Dalton, Ph.D., M.D., and Bausch Health Companies Vice President for Government Affairs Brian Munroe will continue to serve on the Executive Committee as past chairs.

"PMC continues to maintain a Board of Directors with leaders from all sectors of the health care system, each of whom is dedicated to collaborative efforts that advance personalized medicine," said PMC President Edward Abrahams. "By strategically positioning key representatives as Board officers and establishing chair positions for important PMC committees in 2019, the Board has strengthened PMC's position as an educational, advocacy and evidence development organization committed to supporting investment in and adoption of personalized medicine."

"By strategically positioning key representatives as Board officers and establishing Board chair positions for important PMC committees in 2019, the Board has strengthened PMC's position as an educational, advocacy and evidence development organization."



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MEDIA BRIEF

From the PMC News Desk

Following Agency's Announcement of First "Coverage With Evidence Development" Decision for FDA-Approved Anti-Cancer Treatment, PMC Commits to Working With CMS to Ensure Patient Access to Personalized CAR T-cell Therapies

Following the U.S. Centers for Medicare and Medicaid Services (CMS) proposal of "coverage with evidence development" (CED) for an important group of personalized treatments called chimeric antigen receptor (CAR) T-cell therapies in February, PMC committed to working with CMS to ensure patient access to the therapies. The agency's proposal marks the first time that the agency has proposed CED for an anti-cancer therapy that has U.S. Food and Drug Administration (FDA) approval for use in the target population.

"PMC is further evaluating the full impact of CMS' proposed decision memo, but it is our hope that if CMS moves forward with a CED requirement, they will incorporate feedback received from our members, who represent innovators, patients, providers and payers, to ensure that Medicare beneficiaries facing poor prognoses and their health care providers will not suffer from delays in coverage of CAR-T therapies," PMC Senior Vice President for Public Policy Cynthia A. Bens said.

See *FierceHealthcare*: "CMS Looks to Expand Medicare Coverage to CAR-T Therapy" (February 2019)

PMC Analysis Shows Personalized Medicines Topped 40 Percent of New Drugs Approved by FDA in 2018

PMC's *Personalized Medicine at FDA: A Progress & Outlook Report*, published in February, demonstrates that personalized medicines topped 40 percent of new drugs approved by the U.S. Food and Drug Administration (FDA) in 2018. With personalized medicines now accounting for one of every three new FDA approvals, the report underlines the importance of supportive regulatory and reimbursement policies.

"Future progress cannot be taken for granted," the report reads. "To ensure that industry leaders continue to develop groundbreaking personalized medicine tests and treatments and that patients have access to these products, policymakers must favor policies that encourage the advancement of the field."

See *FierceHealthcare*: "FDA Approved Record Number of Personalized Medicines in 2018: Report" (February 2019)

National Alliance for Hispanic Health, PMC Publish Op-Ed Calling for More Diverse Clinical Trials That Can Underpin Personalized Medicine

Underlining the need for more diverse clinical trials that can underpin personalized medicine, National Alliance for Hispanic Health President and CEO Jane L. Delgado, Ph.D., M.S., teamed up with PMC President Edward Abrahams to publish an opinion piece in *STAT News* in January titled "Diversity in Clinical Trials Defines Good Science and Better Medicine."

Delgado and Abrahams note in the article that although "all agree on the worthy goal" of more inclusive clinical trials, more than 80 percent of the genome-wide association studies that could otherwise facilitate personalized prevention and treatment strategies have been conducted among individuals of European descent.

See *STAT News*: "Diversity in Clinical Trials Defines Good Science and Better Medicine" (January 2019)

Industry Leaders Bolster Prospects for Personalized Medicine With Flurry of Investments in Precision Oncology

Industry leaders bolstered the scientific prospects for personalized medicine in January with a flurry of investments in personalized tests and treatments for cancer patients.

Bristol-Myers Squibb led the way with its \$74 billion acquisition of Celgene. BMS CEO and Chairman Giovanni Caforio, M.D., was quick to express his enthusiasm for what he described as a "science-and-pipeline deal" that promises to accelerate BMS' efforts to pursue personalized oncology treatments that home in on the molecular abnormalities that are thought to promote the growth of cancer cells.

The scientific promise of precision oncology also underpins the latest moves from GlaxoSmithKline and Eli Lilly, which recently purchased Tesaro and Loxo Oncology, respectively. Both Tesaro and Loxo are focused almost exclusively on developing personalized medicines in oncology.

On the diagnostics side, Qiagen announced that it will acquire N-of-One, a company that provides clinical interpretation services for oncologists. And Circulogene, a company developing liquid biopsy tests

to guide cancer treatments, has begun developing a test that could help identify which patients may benefit from Loxo's Vitrakvi (larotrectinib).

Summing up these developments, GSK Chief Scientific Officer and R & D President Hal Barron, M.D., said his company's moves in the space reflect the fact that the science underpinning personalized oncology promises tremendous benefits for patients and health systems.

"There is an enormous amount of science that is evolving, and this is just the tip of the iceberg," Barron said.

See *The Wall Street Journal*: "Bristol-Myers Squibb to Acquire Celgene for About \$74 Billion"

See *STAT News*: "GSK Buys Cancer Biotech Tesaro for \$5.1 Billion"

See *The Wall Street Journal*: "Eli Lilly to Buy Loxo Oncology in \$8 Billion Deal"

See *GenomeWeb*: "Qiagen Acquires N-of-One to Add Molecular Decision Support"

See *GenomeWeb*: "Circulogene Broadens Push into Companion Diagnostics With NTRK Fusion Assay" (January 2019)

In Proposal That May Sideline Personalized Medicine, Trump Administration Announces Effort to Tie US Payment Rates for All Therapies to Lower Rates Established by Other Countries

In a proposal that would likely discourage investment in personalized medicine, the Trump administration announced in October an effort to tie the rates that the U.S. government pays for various therapies to lower rates established in other developed countries, without considering the value that each therapy may offer to patients and society.

Because these countries establish lower payment rates by employing economic value assessment methodologies that cannot adequately account for the benefits of an emerging wave of personalized treatments with long-lasting benefits, critics contend that the so-called international pricing index (IPI) would discourage biopharmaceutical companies from developing these treatments. In an interview for a story about developments in personalized medicine in 2018, PMC President Edward Abrahams told *GenomeWeb* that the IPI proposal, like several others touted by the Trump administration, "flies in the face of personalized medicine."

See *GenomeWeb*: "Personalized Medicine in 2018: More Drugs, Greater NGS Adoption, Growing Appreciation of Dx Value" (December 2018)

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