

PERSONALIZED MEDICINE IN BRIEF

VOL. 6, SPRING 2016

Developments in Brief

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Reaching a New Era

by Edward Abrahams, Ph.D., PMC President



As Daryl Pritchard, Ph.D., PMC Vice President for Science Policy, writes in this issue of *Personalized Medicine in Brief*, 2015 was indeed the year that personalized medicine took off. Whether or not it will come to represent the “inflection point” that changes the way we think about modern medicine, which many have been waiting for, only time will tell.

But the facts are these. A Precision Medicine Initiative launched by President Obama in January of 2015 has led to new federal funding for research, a plan to build a national cohort of patients to study individual variation and response, and, on the commercial side, a record number of FDA approvals of new drugs with biomarker strategies in their labels.

These facts are encouraging, but there is no shortage of challenges that remain. Public policies in the space between

the science and the patient make a huge difference in determining the pace of progress away from one-size-fits-all/trial-and-error medicine and toward an era in which the right patient gets the right therapy at the right time.

That is why PMC was created and why we continue to ask for your support.

PMC remains focused on strategic priorities identified by its members, which include finding solutions to the issues facing personalized medicine, spearheading regulatory reforms that accelerate the development of personalized medicine products and services, and championing coverage and payment policies that support the field. These pages contain updates along those lines.

On the topic of regulatory reform, Amy M. Miller, Ph.D., PMC Executive Vice President, analyzes the challenges facing the diagnostic industry as it works to define a regulatory framework that protects patients without stifling innovation and points out lingering concerns that many have expressed about FDA’s proposed guidance on laboratory-developed tests. She has been invited to work with the diagnostic community to determine whether it can come together to agree on a single alternative to FDA’s guidance. You can find a side-by-side comparison of four proposals currently under consideration on pages 6–7.

Also on the regulatory front, a guest column written by David A. Shaywitz, M.D., Ph.D., Chief Medical Officer, DNAexus, explains how a pilot platform launched by his company proposes to give FDA the tools to regulate and thereby integrate next-generation sequencing (NGS) into health care. He notes that the platform addresses three issues: determining the analytic validity of NGS tests, facilitating the evolution of standards to guide decision-making and putting in place a public-private partnership so that FDA can keep up with the rapidly developing field.

For the latest on the ongoing debate about coverage and payment of diagnostic tests, see PMC Communications



President Obama participates in a panel discussion during the “Precision Medicine Initiative Summit” on February 25, 2016. The Precision Medicine Initiative has put a national spotlight on the personalized medicine field.

There has emerged an increased focus on the power of precision or personalized medicine to contribute to medical science by bringing together therapy and diagnosis.

Director Christopher Wells' summary of the call for partnerships among industry sectors to determine which technologies work best to produce desired outcomes, which Foundation Medicine CEO Michael Pellini, M.D., espoused at the 11th Annual Personalized Medicine Conference at Harvard Medical School.

And finally, in our effort to continue to fan the flames of interest in personalized medicine, see the discussion of Vice President Biden's "moonshot" initiative to "cure cancer once

and for all," also by Christopher Wells. That initiative has sparked an interest in what the government can do to stimulate more effective research strategies, and we hope other reforms as well.

As Wells writes in that analysis, there has emerged an increased focus on the power of precision or personalized medicine to contribute to medical science by bringing together therapy and diagnosis.

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As a Personalized Medicine Coalition member, you won't want to miss this important event.

For event updates, please contact Meghan Burton at mburton@foley.com to join our email list.

Preparing for Inevitable? FDA's Regulation of LDTs

by Amy M. Miller, Ph.D., PMC Executive Vice President



Editor's note: The content in this story is complemented by the "Lay of the Lab" table that follows, which was adapted and republished with permission from GenomeWeb.

More than 18 months after FDA first published a detailed proposal for regulating laboratory-developed tests (LDTs), many in the community are still advocating for different approaches to the issue. Pain points remain. But now might be the time to begin considering what the community may ask of FDA in the event that a final guidance is published anyway.

FDA and the Centers for Medicare & Medicaid Services (CMS) have been committed to FDA's original proposal and its intent to finalize an updated version of it since the beginning, and that hasn't changed.

In late November 2015, FDA Center for Devices and Radiological Health Director Jeffrey Shuren, M.D., J.D., took the stage with CMS Chief Medical Officer Patrick Conway, M.D., to discuss possible changes to the regulation of LDTs with the Energy and Commerce Committee in the U.S. House of Representatives.

The agencies were in lockstep as they outlined a single joint strategy. Conway clearly stated that CMS lacked the staff, resources and inclination to regulate LDTs —echoing a statement made nearly a decade earlier, under the previous administration, to the Secretary's Advisory Committee on Genomics, Health and Society.

Shuren, on the other hand, told Congress that FDA is ready, able and willing to take on the mission outlined in the draft guidance. Shuren was also clear and confident that the finalized documents could be expected early in 2016. That confidence corresponds with the argument that in order to comply with the Congressional Review Act's language on regulation under a new administration, President Obama

must finalize them 60 legislative days before the end of his presidency. Add 60 days for the Congressional notice provision of the Food and Drug Administration Safety and Innovation Act (FDASIA) and the finalized documents could have come as early as mid-March.

For many years, various groups contemplated legislative alternatives to both the current device regulation process and FDA's regulation of LDTs. Comparison and discussion of those ideas has recently turned toward areas of commonality, not differences. For example, they all take a tiered approach to regulation, with the high-risk tests getting the most attention from FDA, and in some cases, the only attention. Furthermore, most in the community recognize that FDA's device regulations do not fit diagnostic tests. Finally, many in the community now agree that FDA should be more flexible in its management of modifications, to fit the now-recognized evolution of tests. (The "Lay of the Lab" table that follows this article offers a side-by-side comparison of four alternative LDT regulation proposals.)

Why is so much attention paid to this issue?

First, LDT regulation would put a new burden on FDA. Rather than review currently marketed tests, many in the kit manufacturing industry would like FDA to focus on getting new diagnostics to market, and there is concern that FDA's focus on lab tests would distract the agency from that pursuit.

Second, regulation would put a financial burden on clinical laboratories. Clinical laboratories range greatly in size, and the financial burden of FDA regulation is more than simply user-fees. Labs must manage internal processes and staffing to conform to regulations. Furthermore, they will either need to hire personnel or contract with them to manage filings. These costs, in an ordinary market, would be passed on to the consumer. However, diagnostic coverage and payment policy in the U.S. has led to prices that are so low many labs will not be able to make the investments

All of this clearly shows that the change will be drastic for clinical laboratories. That is why implementation requests to FDA are so important.

in staff needed for FDA regulation. This could result in a substantial contraction of the industry.

Third are the effects on patient access. Significant contraction in the industry will unintentionally hinder access to tests. Furthermore, if the costs of regulation are added to the cost of tests but insurers do not adjust their payment rates, then those cost increases might be passed on to consumers. As a result, patients may avoid testing.

All of this clearly shows that the change will be drastic for clinical laboratories. That is why implementation requests to FDA are so important. How is the agency planning to integrate and assimilate LDTs into a new regulatory paradigm? FDA has long been clear that the proposal starts with high-risk tests, but is it possible to delay the start of that time clock until FDA publishes a priority list and labs come to better understand the definition of a high-risk test? Finally, clinical labs do not fully appreciate FDA's quality

systems requirements and how they conflict or conform to the Clinical Laboratory Improvement Amendments (CLIA) program. Might FDA and CMS provide more technical assistance on that issue?

Recent estimates indicate that there are more than 60,000 gene tests on the market. Of those, nearly 8,000 would probably be considered "high-risk" by FDA, depending on various assumptions. That is only a subset from about 300 labs. Even though the number does include FDA cleared and approved kits, it is still an underestimation of the entire world of high-risk tests.

It is important that FDA builds capacity before taking regulatory action. The agency has a responsibility to prepare labs for this change and provide adequate time for compliance.

FDA's intent is to ensure that personalized medicine testing is safe and effective for patients. Now might be the time to suggest ways to navigate this regulatory shift.



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THE LAY OF THE LAB: A SIDE-BY-SIDE COMPARISON OF FOUR LAB TEST REGULATION PROPOSALS

	FDA	Diagnostic Test Working Group
What is being regulated?	Lab-developed tests (LDTs): in vitro diagnostic tests that are designed, manufactured and used within a single lab	In vitro clinical tests (IVCTs): diagnostic test kits (referred to as “finished products”) and lab test protocols intended by the developer for use in the collection, preparation, analysis or in vitro clinical examination of specimens from the human body in the context of a disease or condition
Which agency is in charge?	<ul style="list-style-type: none"> · FDA extends its oversight for all LDTs in a three-tier, risk-based framework · Third-party reviewers review 510(k)s for lower risk LDTs · CMS forms a task force with FDA to ensure labs won’t have duplicative requirements 	<ul style="list-style-type: none"> · FDA regulates IVCT design, development, validation, platform manufacturing and preparation of reagents for use in more than one CLIA lab or third party · CMS keeps jurisdiction over typical lab activities, such as preparing reagents used at a single lab, developing lab operating procedures, etc. · The states continue to look after interpretation of test results
High risk: Definition	Class III: high-risk tests regulated under general controls and usually requiring premarket approval [The FDA has said it will issue additional guidance on risk classifications and form advisory panels]	An IVCT for a serious or life-threatening disease or disorder that is the sole determinant for directing or changing treatment, where the wrong result has a high risk of serious health injury and the test is not well characterized
Moderate risk: Definition	Class II: moderate- to high-risk tests regulated under general and special controls and usually requiring 510(k)	An IVCT that would be high risk, but is well characterized and is unlikely to have a serious health impact due to a wrong result; or IVCTs that are not well characterized, not the sole determinant for directing or changing treatment; and where wrong results may cause a serious health injury
Low risk: Definition	Class I: low- to moderate-risk tests regulated under general controls, and usually exempt from premarket 510(k) notification	An IVCT that carries a risk of serious injury due to a wrong result but is not the sole determinant for directing or changing treatment and is well characterized; or an IVCT where a wrong result doesn’t have a serious or life-threatening impact
Years to implement?	9	3 – 4

Association for Molecular Pathology	College of American Pathologists
<p>Lab-developed testing procedures (LDPs): testing procedure or service performed in a single CLIA-certified lab, where the development, validation, monitoring, quality assurance, continuous improvement, performance and interpretation of the results of that procedure/ service are conducted</p>	<p>Lab-developed tests (LDTs): lab procedure that is intended to be designed, manufactured and performed in a single, CLIA-certified lab</p>
<ul style="list-style-type: none"> · CMS regulates LDPs under CLIA considered low, moderate and high risk, develops minimum standards, develops continuously updated, searchable database with information on high- and moderate-risk LDPs and establishes a process for approving third-party reviewers · CMS or designated third parties review proprietary LDP data, but labs can choose not to disclose proprietary data and instead submit their tests to FDA 	<ul style="list-style-type: none"> · FDA regulates high-risk LDTs but not moderate- or low-risk LDTs— would require amending Federal Food, Drug & Cosmetic Act · CMS under CLIA regulates moderate- and low-risk LDTs, but not high-risk LDTs— would require amending CLIA provisions of the Public Health Service Act
<p>An LDP for diagnosis, predicting risk or estimating prognosis of a disease that is associated with significant morbidity or mortality; and which includes methodologies such as proprietary algorithms, for which test results cannot be directly tied to analytical data or subjected to inter-laboratory comparisons</p>	<p>An LDT that produces a result that is not independently verifiable and the consequences of an incorrect result or interpretation include a high risk of serious morbidity/mortality</p>
<p>An LDP for diagnosis, predicting risk, estimating prognosis of or predicting therapeutic response for a disease that is associated with significant morbidity or mortality and for which the test methodology lends itself to inter-laboratory comparisons or proficiency testing</p>	<p>An LDT producing an independently verifiable result and the risk of serious injury, morbidity or mortality due to an incorrect result or interpretation is moderate or high</p>
<p>An LDP that isn't appropriately used as the sole determinant of diagnosis, prognosis or therapy selection; or for which an incorrect LDP result is unlikely to result in morbidity or mortality; LDPs for rare diseases, public health emergencies, infectious agents that are not serious public health threats</p>	<p>An LDT producing an independently verifiable result and the risk of serious morbidity or mortality due to an incorrect result or interpretation is low</p>
<p>4</p>	<p>2</p>

Seizing Momentum: Reflecting on the ‘Year of Personalized Medicine’

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



The events over the past year led PMC to label 2015 the “year of personalized medicine.”

The year began with Obama’s announcing the Precision Medicine Initiative (PMI) and a proposal to increase the resources available at the National Institutes of Health and FDA to achieve the PMI’s goals. We also saw significant movement in the laboratory-developed test (LDT) regulatory oversight debate, the introduction of the 21st Century Cures Act in the House of Representatives, efforts to advance the corresponding Innovation for Healthier Americans legislation in the Senate and major partnership deals being struck between diagnostic and pharmaceutical companies. By the end of the year, FDA’s Center for Drug Evaluation and Research (CDER) approved 45 novel new drugs (NNDs), 13 of which — more than 25 percent — were personalized medicines as classified by PMC. Thus, more new treatments and tests for personalizing care were made available in 2015 than in any other year.

As the dust settles on this extraordinary flurry of activity, the pharmaceutical industry is poised for continued progress. A recent study conducted by the Tufts Center for the Study of Drug Development showed that 42 percent of the drugs in the development pipeline now involve biomarkers in their research and development design, and that biopharmaceutical manufacturers have increased investment in personalized medicine product development by 87 percent over the past five years. These companies expect investment to increase by another 33 percent over the next five years.

Nowhere is the transformation of health care toward personalized medicine more clear than in oncology, where the identification of the molecular drivers of specific tumors has enabled therapies targeted to an individual’s disease. The development of new personalized cancer treatment options has outpaced all other disease types, and continues to accelerate. According to the Tufts study, 73 percent of oncology drugs in the pipeline are potential personalized

A NEW ERA

Number of personalized medicines approved at FDA by year



All of this demonstrates the transformation of personalized medicine from an emerging idea a decade ago to an established approach to treating many diseases today. But seizing this momentum will require action from stakeholders throughout the entire health care spectrum.

medicines. Of the 13 personalized NNDs approved in 2015, five are oncology drugs. Additionally, a number of significant new indications for previously existing cancer drugs were approved, including Iressa (gefitinib), Opdivo (nivolumab) and Keytruda (pembrolizumab). These new indications redefine and expand the target populations for these drugs, thereby providing more patients with more effective personalized treatment options.

All of this demonstrates the transformation of personalized

medicine from an emerging idea a decade ago to an established approach to treating many diseases today. But seizing this momentum will require action from stakeholders throughout the entire health care spectrum.

PMC is committed to that future, and continues to work toward it with initiatives that facilitate the integration of personalized medicine into health care systems, provide education about personalized medicine and encourage public policies that support the field.



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**KNEED MEDIA**

Field Adopts Sense of Urgency, Champions Collaboration

by Christopher Wells, PMC Communications Director



Many of the attendees at the 11th Annual Personalized Medicine Conference in November agreed on the need for collaborative initiatives that accelerate the pace of progress in personalized medicine, and some of its participants are now spearheading efforts toward that end.

These participants include New England Patriots owner Robert Kraft and Foundation Medicine CEO Michael Pellini, M.D.

Robert Kraft Invests \$20 Million to Accelerate Progress

New England Patriots owner and philanthropist Robert Kraft helped cap a historic year for personalized medicine on the first day of the conference by joining President Obama and Vice President Biden in pledging support for the field, announcing a donation of \$20 million to Harvard Business School (HBS) for the purpose of facilitating collaboration between researchers, the pharmaceutical industry and investors. Kraft said the decision was driven in part by the loss of his wife, Myra Kraft, to ovarian cancer in 2011.

“We didn’t get early detection, and she went through seven rounds of chemo,” Kraft said, reflecting on his wife’s experiences during 15 months of treatment. He said he believes discoveries in personalized medicine are not reaching patients fast enough, and that collaboration is the key to addressing that issue.

The gift establishes an endowment at HBS to accelerate progress. The school has wasted no time in getting started.

HBS launched the Precision Trials Challenge in January, through which the school is inviting proposals for clinical trial designs that can bring personalized diagnostics and therapies to market faster. A panel of judges will select one

winner and two runners-up to share a \$100,000 prize. The author of the winning submission will also have an opportunity to present at the 12th Annual Personalized Medicine Conference at Harvard Medical School, which is scheduled for November 15 – 17, 2016.



Guided by a powerful sense of urgency following the loss of Robert’s wife, Myra, to ovarian cancer in 2011, the Robert and Myra Kraft Family Foundation has established a \$20 million endowment for the advancement of personalized medicine at Harvard Business School. Pictured here from left to right are Robert’s son Jonathan, Harvard Business School’s Richard Hamermesh, Ph.D., Robert Kraft, Harvard Medical School’s Todd Golub, M.D., and Harvard Business School’s Robert Huckman, Ph.D.

“How can we develop business models that support the advancement of precision medicine? How can we get new therapies to market faster and at a lower cost? Our Precision Trials Challenge will help answer these questions by encouraging conversation and helping to put leading-edge ideas into practice.”

Harvard Business School Professor Richard Hamermesh, Ph.D.,
on the impact of Robert Kraft’s \$20 million investment in personalized medicine

“Many of the big challenges facing precision medicine today are actually business challenges,” said HBS Professor Richard Hamermesh, Ph.D. “How can we develop business models that support the advancement of precision medicine? How can we get new therapies to market faster and at a lower cost? Our Precision Trials Challenge will help answer these questions by encouraging conversation and helping to put leading-edge ideas into practice.”

Michael Pellini, Foundation Medicine Embrace Collaboration

On behalf of Foundation Medicine, CEO Michael Pellini, M.D., echoed Kraft’s calls for collaboration on the second day of the conference, two months before Foundation announced an innovative partnership with a health insurance company in New Jersey.

During a panel discussion with other innovators in personalized medicine, Pellini stressed the fact that accelerating the pace of progress will require partnerships that erode boundaries between industries.

“There is incentive to work together to improve care,” Pellini said. He went on to add that collaborations around patient access are of particular importance. In January, Foundation Medicine announced such an effort.

The company has partnered with Horizon Healthcare Services, New Jersey’s oldest and largest health insurer. Foundation will work with Horizon to study the clinical and economic benefits associated with the use of its FoundationOne genomic profiling assay. Horizon has indicated that it may provide its members with coverage for the test as part of metastatic lung cancer evaluations if the results of the study demonstrate its value. Foundation Medicine Chief Medical Officer Vincent Miller, M.D.,



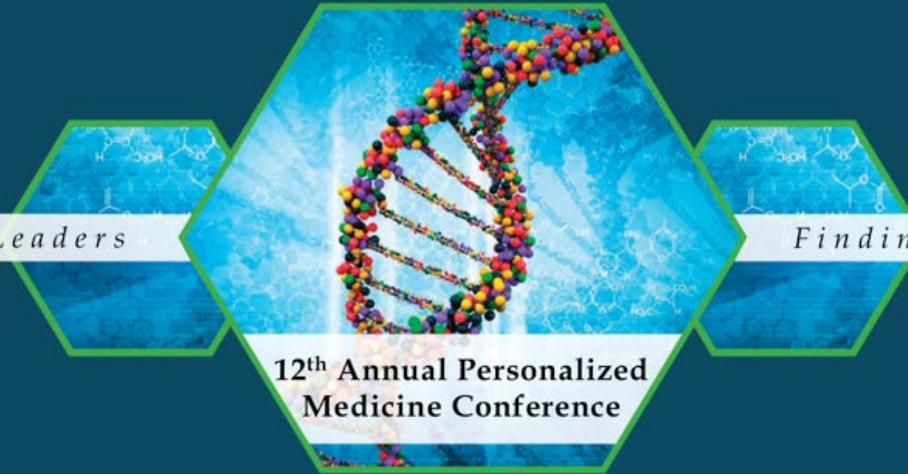
Foundation Medicine CEO Michael Pellini, M.D., who is pictured here leading his panel discussion with Regeneron Laboratories President George Yancopoulos, M.D., Ph.D. (left), Siemens Healthcare Diagnostics CEO Michael Reitermann (center) and N-of-One CEO Christine Cournoyer, called for increased collaboration during the 11th Annual Personalized Medicine Conference. Two months later, Foundation Medicine announced an innovative partnership with Horizon Healthcare Services.

said he believes the effort is a clear example of the value of partnerships.

“We believe this study design... represents an innovative model of collaboration that will catalyze the consistent integration of comprehensive genomic profiling in clinical care for the treatment of metastatic lung cancer,” Miller said. “We fully expect the study to demonstrate the clinical and health economic benefits of a comprehensive approach.”

Innovative Leaders

Finding Solutions



**12th Annual Personalized
Medicine Conference**

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NOVEMBER 15 – 17, 2016

JOSEPH B. MARTIN CONFERENCE CENTER
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The Annual Personalized Medicine Conference has attracted participation from many of personalized medicine's most established leaders, including several of the scientists who contributed to the first complete sequencing of the human genome, some of the world's most successful investors and two FDA commissioners. This year's conference will build on that tradition, but with a more concentrated focus on generating solutions to the field's challenges.

Like its predecessors, the 12th Annual Personalized Medicine Conference will explore new and transformational insights related to the science, business and policy trends impacting personalized medicine. The conference will showcase what is new, offer a

deep dive into the issues, and provide partnership and networking opportunities for attendees. But most importantly, it will propose ways to encourage investment in innovation and foster the adoption of personalized medicine in the clinic.

All those with an interest in personalized medicine, including physicians, scientists, investors, policymakers, patients, and representatives from the pharmaceutical, diagnostics, health insurance, biotechnology and information technology industries, are invited to attend the event, which will take place in the Joseph B. Martin Conference Center at Harvard Medical School from November 15 – 17, 2016.

SPONSORSHIP OPPORTUNITIES

Sponsorship opportunities for the 12th Annual Personalized Medicine Conference are available. Please contact Mary Bordononi at mbordononi@personalizedmedicinecoalition.org for more information.

Biden Spearheads ‘Moonshot’ Initiative

by Christopher Wells, PMC Communications Director

“For the loved ones we’ve all lost, for the families that we can still save, let’s make America the country that cures cancer once and for all.”

President Barack Obama, January 13, 2016

In a blog posted shortly after these words from the President’s State of the Union Address, Vice President Joe Biden said the new “moonshot” initiative seeks to “end cancer as we know it” by accelerating progress in “immunotherapy, genomics and combination therapies.” Although the exact nature and ultimate impact of the ambitious undertaking remain to be seen, stakeholders say Biden’s impassioned effort reflects the extraordinary pace of progress in personalized medicine research.

“Now is the time for a major new initiative in cancer science that supports and builds upon our basic science foundation while translating these exciting scientific discoveries into improved treatments for cancer patients, such as in the areas of genomics, precision medicine and immuno-oncology,” explained José Baselga, M.D., Ph.D., president of the American Association for Cancer Research (AACR).

The initiative, which follows Biden’s announcement in November of his intent to dedicate himself to accelerating progress in oncology after his term expires, will focus on providing additional resources for cancer research and breaking down silos that prevent sharing of data. Biden lost his son, Beau, to brain cancer in May of last year. Biden’s aides said his decision to commit to the effort was made following a series of meetings with a wide range of stakeholders.

“It really captures so many of the policies and ideals that make the Vice President special,” said Robert Hoopes, a former aide to Biden. “It’s personal. He brings intensity to it that really resonates with people. He knows how to get stuff done.”

The initiative comes at a time when personalized medicine research in oncology is surging. Data released last year by the Tufts University Center for the Study of Drug Development suggest that 73 percent of the oncology drugs

now in development are personalized medicines, and PMC’s latest analysis demonstrates that 35 percent of FDA’s 2015 oncology approvals are personalized medicines. Biden hopes to make unprecedented progress by capitalizing on the momentum, and some stakeholders believe reimbursement policy changes are the way to do that. These advocates are disappointed that the Centers for Medicare and Medicaid Services are not represented on the initiative’s task force, which Obama formalized in late January.

“We need our payment policy to keep up with the science,” PMC Executive Vice President Amy M. Miller, Ph.D., told *STAT News* in January.

But despite concerns about the implementation of the initiative, many, including Biotechnology Innovation Organization (BIO) President and CEO Jim Greenwood, welcome the announcement of a government initiative to help spur progress.

“The process of discovering new cures and treatments for patients in need relies upon an ecosystem of innovation in which all stakeholders, including government agencies and regulators, academic research institutions, private industry and patient groups, play a critical role,” Greenwood said.

Biden’s recommendations to accelerate progress are due to the President by the end of the year.



Vice President Joe Biden

GUEST ESSAY

Why precisionFDA Matters

by David A. Shaywitz, M.D., Ph.D., DNAnexus Chief Medical Officer



In December 2015, FDA announced the launch of the pilot precisionFDA platform, described by its key agency champions, Taha Kass-Hout, M.D., M.S., and Elaine Johanson, as “an online, cloud-based portal that will allow scientists from industry, academia, government and other partners to come together to foster innovation and develop the science behind a method of ‘reading’ DNA known as next-generation sequencing (NGS).”

The platform, which was developed under the terms of a contract with DNAnexus, was established to help advance the regulatory science needed to assess the accuracy of genome tests and software. Currently, the value of secondary analysis is undermined when datasets and bioinformatics tools are not harmonized for comparison and reproducibility. By providing a secure, cloud-based platform that is open and transparent to the genomics community, precisionFDA enables researchers and test developers to explore NGS methodologies in order to spur innovation needed to develop necessary standards, which will ultimately improve the evaluation process through consistent data quality, increased integration and reproducibility, and improved data exchange with collaborators.

I’m especially excited by the precisionFDA initiative, for several reasons.

First, it addresses an important problem in the field: the analytic validity of NGS tests. The ready availability of relatively inexpensive sequencing has enabled us to contemplate diagnostic sequencing at a scale that would have been difficult to imagine even a decade ago. At the same time, the drive to apply sequencing in different clinical contexts raises a critically important question: do I trust this test?

A key starting point for clinical interpretation of DNA data is to agree on the sequence itself. If your procedure and analysis reports that a particular sequence in a DNA sample is “GATCGATC” and my procedure and analysis of the same DNA says the sequence is “GATTGATC,” then we’ve got a problem. PrecisionFDA will allow users to compare approaches and determine where refinements might be needed.

Second, precisionFDA represents a novel and forward-thinking approach to regulation. Rather than envisioning governmental regulators as the folks who will define and then impose a specific set of performance standards, precisionFDA sees the government as providing the platform that will enable the NGS community to evolve the standards on their own — organically and transparently.

Finally, the ability to design, refine and deploy this platform in such a rapid and agile fashion reflects in part the value of well-conceptualized public-private partnerships, in this case between FDA and DNAnexus.

The ultimate success of the precisionFDA platform will of course depend upon how well it serves the community it is intended to support. However, it’s hard to think of a more auspicious beginning, and I am hopeful that success here will encourage more leaders to consider addressing important shared challenges through public-private partnerships that deploy platforms designed to leverage the power of a distributed innovation community.



FDA’s recently unveiled online platform for analyzing next-generation sequencing data, pictured here, allows community members to conduct genome analyses for comparison against reference material.

THE PERSONALIZED MEDICINE COALITION PRESENTS

The 12th Annual State of Personalized Medicine Luncheon Address

Wednesday, May 25, 2016 · 12:00 – 2:00 p.m. ET

The National Press Club

Washington, D.C.



FEATURING A KEYNOTE
ADDRESS BY

Stephen J. Ubl
President & CEO, PhRMA

The Annual State of Personalized Medicine Luncheon Address provides a forum for PMC members and guests from the health care community to engage policy leaders in a discussion of the key issues facing the field.

Email David Davenport at ddavenport@personalizedmedicinecoalition.org to register.

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White House Announces Next Steps for Precision Medicine Initiative

by Christopher Wells, PMC Communications Director

On February 25, the Obama administration laid out the next steps for the Precision Medicine Initiative (PMI) during the “Precision Medicine Initiative Summit” at the White House. Zeroing in on President Obama’s vision of building a research cohort that includes one million Americans, the National Institutes of Health (NIH) expect to enroll 79,000 people by the end of 2016. The effort also includes plans to encourage data sharing and start developing standardized applications that will make it easier for people to contribute data, which is expected to, in turn, inform research.

While participating in a panel discussion at the summit, President Obama stressed the importance of sharing personalized medicine data with consumers.

“[You may have] genetic variants that alter how you think about your blood pressure, your likelihood for diabetes, a whole range of potential markers,” Obama said. “If we do precision medicine well, and we get that information and that data to consumers, it gives them the ability to stay healthy for long periods of time. And that’s hugely promising.”

In addition to announcing its own next steps for the initiative, the White House also highlighted the plans of more than 40 private organizations that have pledged their support of the effort. These organizations are led by Vanderbilt University and Verily, which was formerly known as Google

Life Sciences. As partners on the NIH’s Direct Volunteers Pilot Studies program, Vanderbilt and Verily will work with the NIH to establish and test innovative methods and technologies for engaging volunteer patients. The lessons learned will be used to inform the NIH’s future work with the volunteer cohort.

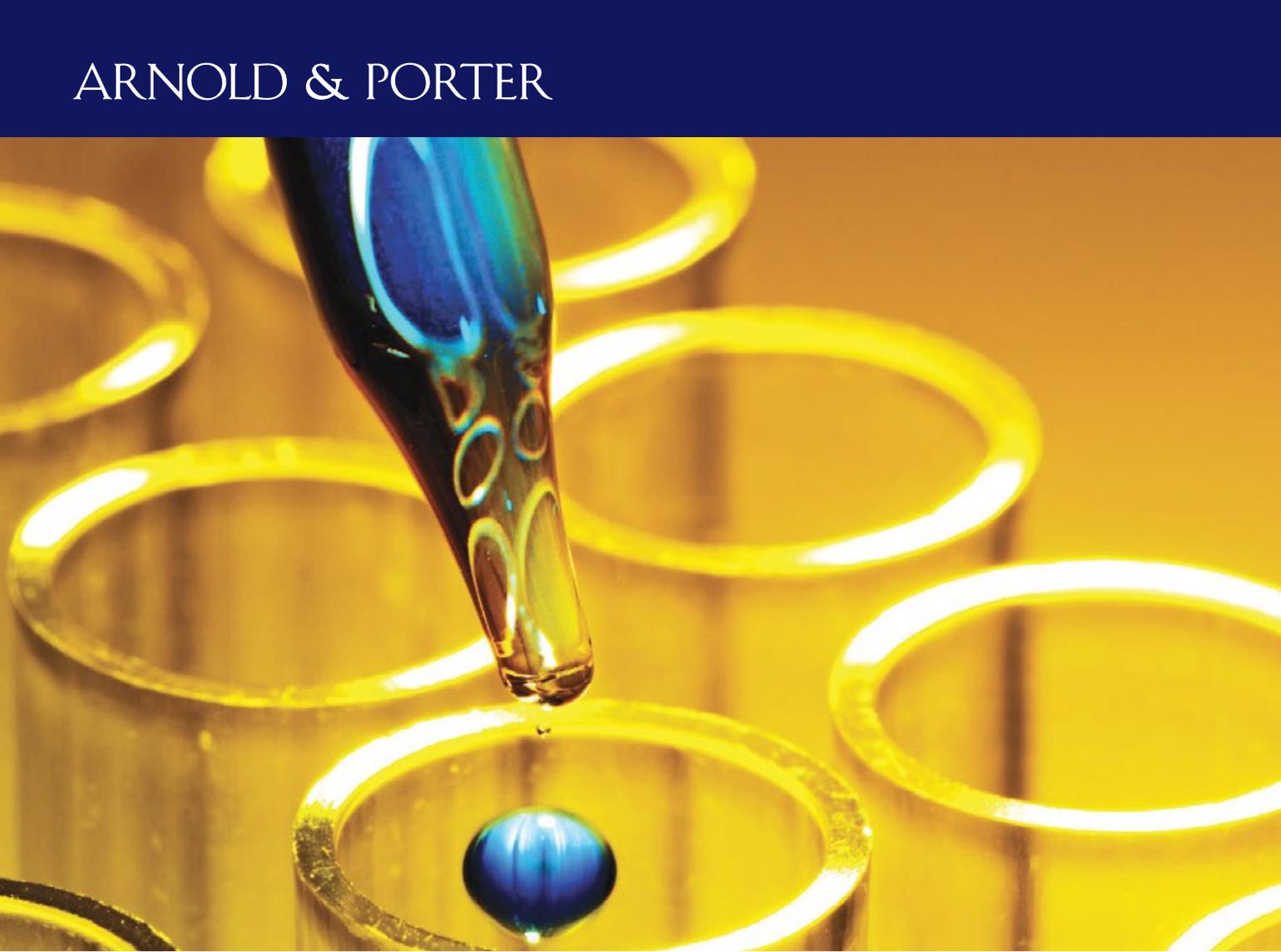
“This approach will help us learn how to create durable relationships with volunteers, who are partners in the research process, which will be the foundation for a democratized, transformative research environment,” NIH Director Francis Collins, M.D., Ph.D., said in a statement.

PMC Executive Vice President Amy M. Miller, Ph.D., who attended the White House event, is encouraged by the administration’s willingness to work with the private sector. She said she believes regulatory and reimbursement policies will play a large role in determining the PMI’s ultimate impact.

“The downstream impact of the PMI will ultimately depend on the extent to which U.S. regulatory and reimbursement policies encourage the timely integration of personalized medicine into health care systems,” Miller said in a statement. “We look forward to continuing our engagement with FDA and the Centers for Medicare and Medicaid Services as they evaluate the personalized medicine implications of their policies.”

“If we do precision medicine well, and we get that information and that data to consumers, it gives them the ability to stay healthy for long periods of time. And that’s hugely promising.”

President Obama during the “Precision Medicine Initiative Summit” at the White House



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Arnold and Porter is proud to be a Strategic Partner of the Personalized Medicine Coalition. For more information on our capabilities, visit our website at www.arnoldporter.com or contact our practice group co-chairs Dan Kracov at daniel.kracov@aporter.com or Allison Shuren at allison.shuren@aporter.com.

MEDIA BRIEF

From the PMC News Desk

Ambry Genetics to Make Customers' Data Publicly Available

When Ambry Genetics announced its intent to make data collected from 10,000 of its customers publicly available in March, *The New York Times* called on PMC President Edward Abrahams, Ph.D., to learn more about the implications of the decision. Abrahams told the paper that Ambry's move is "to be applauded," noting that data in silos can pose challenges for personalized medicine.

The company says sharing the data is an effort to further progress in scientific research. Ambry made the information available on a public database called AmbryShare, and to protect patient privacy, the information is available as aggregated data only. The actual exomes of each person are not available. *The New York Times* (March 2016)

Calls Intensify to Get Medicare to Pay for Genetic Sequencing of Tumors

In January, *STAT News* called on PMC Executive Vice President Amy M. Miller, Ph.D., to help explain why a growing number of personalized medicine advocates believe the Obama administration needs to involve itself in the Centers for Medicare and Medicaid Services (CMS)' decisions on reimbursement for gene-sequencing of tumors.

"We have this weird juxtaposition with the White House and the Vice President loving personalized medicine," Miller explained. "And CMS often isn't paying for it."

Miller also pointed out that many personalized medicine diagnostics are proven to benefit patients.

"We need our payment policy to keep up with the science," she said. *STAT News* (January 2016)

FDA Approving Personalized Medicines in Record Numbers

Following the Coalition's release of a report analyzing the number of FDA's 2015 approvals that are personalized medicines, *GenomeWeb* published a story on the agency's record-breaking year. The piece reinforced that the number of personalized medicine approvals has been increasing since PMC's first report on the topic in January of last year.

"The high proportion of new approvals that are personalized medicines demonstrates the progress researchers have made in advancing the field from an emerging idea a decade ago to an established approach to treating many diseases today," PMC's statement reads. *GenomeWeb* (January 2016)

Personalized Medicine Makes List of Top Pharma and Biotech Trends to Watch for in 2016

Industry's efforts to develop targeted therapies were highlighted by the *International Business Times* as a top biopharmaceutical trend to watch in 2016.

The piece cites data from PricewaterhouseCoopers indicating that 94 percent of companies are invested in personalized medicine research. It also includes a statistic from the Coalition's analysis of 2014 drug approvals, which demonstrates that 21 percent of new drug approvals in 2014 are personalized medicines.

The article states that the trend toward personalized medicine is "widely seen as positive for patients." *International Business Times* (December 2015)

FDA Launches precisionFDA, a Sharing Tool for Next-Generation Sequencing

Anticipating the launch of FDA's precisionFDA web platform, *The Gray*

Sheet spoke with PMC Vice President Daryl Pritchard, Ph.D., in the early part of December to learn more about the tool's purpose. Pritchard explained that although the platform is not designed as a regulatory tool, the agency expects it to help inform the extent to which databases could be used in the regulation of next-generation sequencing technologies.

"The overall concept of using databases to build a regulatory-oversight grade system for next-generation sequencing diagnostic tests has been met with a lot of... positive feelings from the diagnostic and pharmaceutical industries, and really the entire community," Pritchard said. *The Gray Sheet* (December 2015)

Case Study Highlights Opportunities for Personalized Medicine in Cardiovascular Disease

In September of last year, a reporter for *NPR* used data published on PMC's blog, *Education & Advocacy*, to highlight the opportunities for personalized medicine in cardiovascular disease. The author uses a personal experience to demonstrate how one personalized medicine company used a genetic analysis to help her understand her risk for side effects associated with statins. She observes that drugs for cardiovascular disease are noticeably absent from the Coalition's lists of recent personalized medicine approvals.

"With the exception of new genetically targeted drugs for chronic hepatitis C, where patients' response depends on the genotype of their infection, all these personalized drugs treat rare diseases and unusual forms of cancer, where disease is caused by one gene or a handful of genes," she writes. *NPR* (September 2015)

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