PMC PERSONALIZED MEDICINE COALITION

PERSONALIZED MEDICINE IN BRIEF

VOL. 8, SPRING 2017

Developments in Brief

2017

PRESIDENT'S BRIEF

New Challenges Emerge for Personalized Medicine

by Edward Abrahams, Ph.D., PMC President

Although President Trump recently acknowledged that health care policy is an "unbelievably complex subject," his insight has not slowed the dizzying set of proposals coming out of the new administration and Congress, many of which could have dramatic implications for the future of personalized medicine.

These proposals, which remind us of the importance of PMC's mission to educate and advocate on behalf of a new medical paradigm, have also led PMC to plot new strategies to protect past progress, as well as redouble our efforts to convince policymakers that the only way we can improve health and make health care more efficient is to target treatments to the right patients at the right time, preferably before they get sick. Or as Sen. Edward Markey (D-MA) said at a PMC Congressional briefing on personalized medicine in March, "work smarter, not harder." By investing in and adopting new technologies and therapies, we can both relieve suffering and reduce overall health care costs. For more information on the briefing, see pages 4–5.

The U.S. House of Representatives Committee on Education & the Workforce, led by Rep. Virginia Foxx (R-NC) (second from right), recently passed the Preserving Employee Wellness Programs Act (H.R. 1313), which would carve out an exemption from a provision of the Genetic Information Nondiscrimination Act (GINA) that prohibits employers from using genetic information in wellness programs. Proponents say the bill would facilitate preventive medicine, while critics argue that it would open the door to genetic discrimination.

Although President Trump recently acknowledged that health care policy is an "unbelievably complex subject," his insight has not slowed the dizzying set of proposals coming out of the new administration and Congress, many of which could have dramatic implications for the future of personalized medicine.

Suffice it to say, we have our work cut out for us to convince the public that this is the case. There is no substitute for developing the evidence that our contention is accurate, as Daryl Pritchard, Ph.D., explains in his Issue Brief on page 10 outlining the goals of a PMC-commissioned study on the clinical and economic utility of next-generation sequencing.

In the meantime, the Trump administration has proposed a $1.2 billion cut in funding for the National Institutes of Health (NIH) this year and a $5.8 billion cut, about one fifth of the total, next year. While most observers do not believe Congress will go along with the proposed cuts, the proposals require that proponents of personalized medicine ensure that the foundation for our aspirations — biomedical research — is not cut from under us.

This will be the context for PMC's 13th Annual State of Personalized Medicine Luncheon Address at the National Press Club in Washington, D.C., on May 2 where our guest speaker will be Eric Dishman, Director of the *All of Us*℠ Research Program at NIH, who will explain the purpose of the proposed plan to build a one million-person cohort for research into how individual variation shapes health and disease. As the head of the highest profile research project at NIH on personalized medicine, his views on the purpose of the program promise to be interesting. For more information about the luncheon, see pages 6–7.

And for an international point of comparison, see the essay on pages 16–17 by Drs. Nahla Afifi and Said Ismail about Qatar's plans to recruit, map and track 60,000 people, about 20 percent of Qatar's total population, to determine if the phenotypic and genetic data that they are collecting, including whole genome sequences, can improve the health of the citizens of that country.

Also possibly significant for the future of personalized medicine is a bill, H.R. 1313, the Preserving Employee Wellness Programs Act, which would carve out an exemption from a provision of the Genetic Information Nondiscrimination Act (GINA), passed in 2008, that prohibits employers from using genetic information in wellness programs. As Christopher Wells writes in his analysis on pages 8–9, the proposal pits not only Republicans against Democrats, but also advocates of genetic privacy against some large employers, who would like to use financial incentives tied to genetic information to encourage a healthier workforce.

Still, as we also show on pages 12–15 in essays on how clustered regularly interspaced short palindromic repeats (CRISPR) may provide treatments for hitherto impossible-to-treat eye diseases and how a new precision oncology drug in development has changed the life of one patient without chemotherapy, the pace of scientific and technological progress in personalized medicine continues to excite patients and their providers, despite the challenges in the public sphere.

NEWS BRIEF

Sen. Markey, Health Sectors Unite in Capitol to Underline Potential of Personalized Medicine

by Christopher Wells, PMC Communications Director

In a PMC-organized effort to keep personalized medicine on the health care agenda as Congress considers its budgetary and health reform priorities, panelists from four different sectors of the health care system joined Sen. Edward J. Markey (D-MA) on March 7 to demonstrate to an audience of Congressional staffers from both sides of the aisle that public policies in support of the field can facilitate better health care while decreasing costs.

The conversations, which took place in the Senate's Kennedy Caucus Room during a briefing titled *Personalized Medicine: Opportunities and Public Policy Challenges,* featured PMC Board members Stephen L. Eck, M.D., Ph.D., Vice President, Oncology Medical Sciences, Astellas Pharma Global Development; Michael Sherman, M.D., M.B.A., M.S., Senior Vice President, Chief Medical Officer, Harvard Pilgrim Health Care; and Jay G. Wohlgemuth, M.D., Chief Medical Officer, Senior Vice President, Quest Diagnostics. Personalized medicine, they explained, targets each treatment to only those patients who will benefit, eliminating the expenses and side effects of prescribing the treatment to those who will not.

But it was Emily Kramer-Golinkoff, M.B.E., who has a form of cystic fibrosis that cannot yet be cured, who articulated the promise of innovation in personalized medicine most directly.

"Personalized medicine is my hope for the future," said Kramer-Golinkoff, who is the Co-Founder of Emily's Entourage.

As PMC's honorary host and keynote speaker, Sen. Markey praised the briefing, which took place just one day before the Senate Appropriations Committee held a hearing on medical research, for exemplifying the importance of research in personalized medicine and other areas. He noted in particular that President Donald Trump's proposal to increase defense spending by $54 billion would be offset by cuts to spending in other areas, including biomedical research. The benefits of biomedical research, he said, should be carefully considered before funding is withheld.

PMC Board Chairman Stephen L. Eck, M.D., Ph.D., explains the principles of personalized medicine to an audience of Congressional staffers and PMC members in the U.S. Senate's Kennedy Caucus Room during a PMC-organized briefing in March.

"Personalized medicine is my hope for the future."

Emily Kramer-Golinkoff, M.B.E., Co-Founder, Emily's Entourage, cystic fibrosis patient

"If they have massive tax cuts plus a massive defense spending increase plus a massive wall [on the U.S.-Mexico border] — and they continue to say they want to balance the budget — well, something has to go," Markey explained to *Bloomberg BNA* after his remarks. "You don't have to be a genius detective to figure out what's left."

Following the discussion of personalized medicine's benefits, the panelists noted that Congress could help unlock the field's potential by addressing ongoing challenges in regulation, reimbursement and clinical adoption. Wohlgemuth encouraged the audience to specifically consider facilitating increased reimbursement rates for genetic tests. Payment rates, he said, should reflect not only the costs of the tests themselves but also the economic value of the data they provide. Those data, he pointed out, can be used to eliminate costs associated with treating patients with therapies from which they derive no benefit.

"[Personalized medicine helps ensure that] people get the correct treatment every time," he explained.

The Coalition provided each attendee at the briefing with a copy of *The Personalized Medicine Report: Opportunity, Challenges, and the Future,* which summarizes the evidence supporting the field's most established benefits. Those benefits include:

- Shifting the emphasis in medicine from reaction to prevention
- Directing targeted therapy and reducing trial-and-error prescribing
- Reducing adverse drug reactions
- Revealing additional targeted uses for medicines and drug candidates
- Increasing patient adherence to treatment
- Reducing high-risk invasive testing procedures
- Helping control the overall cost of health care

From bottom left: Sen. Edward J. Markey (D-MA) stresses the importance of biomedical research in personalized medicine and other areas; Emily Kramer-Golinkoff, M.B.E., a cystic fibrosis patient and Co-Founder of Emily's Entourage, explains the field's significance for patients; the expert panel of speakers settles in as PMC President Edward Abrahams explains that thoughtful public policies are essential to the future of personalized medicine.

NEWS BRIEF

Health Care Community Continues to Advocate for Precision Medicine Initiative

by Christopher Wells, PMC Communications Director

With President Donald Trump proposing drastic cuts to discretionary spending on biomedical research and aggressively pursuing a bullish agenda on immigration, some observers have begun to question whether the National Institutes of Health (NIH)'s *All of Us*℠ Research Program team will have all the pieces in place when it begins building a one million-person cohort of research volunteers that includes historically underrepresented populations. But many in the health care community maintain that the time is right for the *All of Us* program, which is part of the federal government's Precision Medicine Initiative (PMI), and advocates for personalized medicine say the PMI's benefits actually synergize with the Trump administration's goals.

The health care community continues to advocate on behalf of Eric Dishman, Director, *All of Us*℠ Research Program, and his team as they seek to advance the federal government's Precision Medicine Initiative. Dishman is pictured here delivering remarks at the 12th Annual Personalized Medicine Conference at Harvard Medical School last year.

"I think it's the right time," Norma Cuellar, Ph.D., R.N., F.A.A.N., President-elect, National Association of Hispanic Nurses, recently told *GenomeWeb*. "If anything, this needed to be done yesterday. We need to do it because it is difficult."

The PMI emerged as a bipartisan Presidential priority after then-President Barack Obama launched the effort in 2015 to "give us the tools to better understand, prevent and treat everyone's health needs." Congress allocated more than $200 million for the effort in December of that year, and approved an additional $4.8 billion for the PMI, the Brain Research Through Advancing Innovative Neurotechnologies (BRAIN) Initiative and the Cancer Moonshot℠ program through the 21st Century Cures Act after the election in 2017.

Although Trump has thus far been silent on the fate of the PMI, PMC President Edward Abrahams notes that the benefits of personalized medicine include better care and lower costs, which Trump described as two of his top priorities in health care during an interview with *ABC News* in January. The insights from research projects like the *All of Us* program, Abrahams said, will facilitate the expansion of these goals.

> "Research programs like *All of Us* and the Cancer Moonshot, which enjoy bipartisan support in Congress, accelerate the country's drive toward the promise of personalized medicine — improved patient outcomes and a more efficient and cost-effective health system."
>
> Edward Abrahams, President, Personalized Medicine Coalition

"Research programs like *All of Us* and the Cancer Moonshot, which enjoy bipartisan support in Congress, accelerate the country's drive toward the promise of personalized medicine — improved patient outcomes and a more efficient and cost-effective health system," Abrahams said. "They represent bets on America's future."

Meanwhile, *All of Us* Research Program Director Eric Dishman, who won his own battle with a misdiagnosed cancer in part thanks to personalized medicine, remains firmly committed to the program's mission.

"This is the great unknown," Dishman notes. "When we're done, we're going to have data on all of us."

Dishman will discuss the importance of the initiative during his keynote address at PMC's 13th Annual State of Personalized Medicine Luncheon Address, scheduled to begin at 12:00 p.m. ET at the National Press Club in Washington, D.C., on May 2, 2017.

THE PERSONALIZED MEDICINE COALITION PRESENTS

The 13th Annual
State of Personalized Medicine Luncheon Address

Tuesday, May 2, 2017 • 12:00–2:00 p.m. ET
The National Press Club, Washington, D.C.

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.

FEATURING A KEYNOTE ADDRESS BY
Eric Dishman, Director,
All of Us℠ Research Program

Email David Davenport at
ddavenport@personalizedmedicinecoalition.org

SPONSORED BY The Coalition for 21st centurymedicine

ISSUE BRIEF

Lawmakers Weigh Merits of Allowing Wellness Programs to Require Genetic Data From Participants

by Christopher Wells, PMC Communications Director

Passed in 2008, the Genetic Information Nondiscrimination Act (GINA), which provides a foundation for personalized medicine by assuring patients that sharing their genetic data cannot lead to discrimination, indicates that employers in the U.S. may "request, require or purchase" genetic information as part of employee wellness programs only if the employee provides "voluntary authorization." Nine years later, a House bill has ignited fierce debate about whether employers who require employees to provide genetic information to qualify for these programs, which are often associated with significant financial incentives, are violating the spirit of the law.

Rep. Virginia Foxx (R-NC) sponsored the Preserving Employee Wellness Programs Act (H.R. 1313) to "provide regulatory clarity so employers can have the certainty they need to continue offering their workers the option of participating in employee wellness programs." The bill has drawn criticism from genetic privacy advocates, who argue that the bill allows employers to coerce employees into sharing genetic information.

Rep. Virginia Foxx (R-NC) sponsored the Preserving Employee Wellness Programs Act (H.R. 1313), which has passed through the U.S. House of Representatives' Committee on Education and the Workforce that she chairs, to "provide regulatory clarity so employers can have the certainty they need to continue offering their workers the option of participating in employee wellness programs." By explicitly stating that employers are permitted to require genetic information from employees who wish to participate in voluntary wellness programs, proponents say the bill would allow employers to facilitate personalized disease management plans for their employees without fearing that they are violating GINA's provisions. Genetic tests, they say, are comparable to other assessments already required from employees who volunteer to participate in the programs.

"Disease management programs, which are among the most successful wellness programs, often include diagnostic testing and screening for conditions or diseases," Foxx said in a statement posted on March 16, 2017. " … These types of tests and assessments — which only occur after an individual has voluntarily decided to participate in the program — help ensure the program effectively improves the health of those workers who choose to participate."

The law's critics, however, who include the Association for Molecular Pathology (AMP) and the American Society for Human Genetics (ASHG), see it differently. Pointing out that wellness programs offer financial incentives of as much as 30 percent of insurance premiums, they argue that requiring employees to provide genetic information in order to access those benefits coerces the provision of that information and conflicts with GINA's

> "People weigh this very carefully. They are reassured by the protections we tell them about, and they are frightened when protections are missing. This whole thing just adds uncertainty and fear."
>
> Robert C. Green, M.D., M.P.H., Geneticist, Brigham and Women's Hospital

effort to prevent employers from obtaining information that may be used for genetic discrimination. AMP and ASHG joined nearly 70 other organizations in signing a letter to oppose the bill.

"H.R. 1313 would allow penalties up to a maximum averaging many thousands of dollars per year if employees decline to disclose information from genetic tests that they, their spouses, their children or their other family members have had, or if they do not reveal their families' medical histories," the letter reads. "Allowing penalties of this magnitude would clearly allow employers to coerce employees into revealing their private genetic information."

Researchers also point out that participants in genetic research are often unfamiliar with the nuances of GINA and are reassured by the law's nearly universal protections. Adding more caveats to GINA's protections, they say, could make it difficult for scientists to recruit participants in important new studies.

"People weigh this very carefully," said Robert C. Green, M.D., M.P.H., who regularly conducts personalized medicine studies in his role as a geneticist at Brigham and Women's Hospital. "They are reassured by the protections we tell them about, and they are frightened when protections are missing. This whole thing just adds uncertainty and fear."

Experts say Senate opposition to the bill, which legislators in the Committee on Education and the Workforce passed along party lines, will likely stifle its chances of becoming law.

ISSUE BRIEF

Encouraged by Field's Growth, PMC Seeks to Explore Economic Utility of Next-Generation Sequencing

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

In many ways, the field of personalized medicine has turned a corner on its path to reshaping medical practice. For example, PMC counted 132 personalized medicines on the market in 2016, compared with just five in 2008. With this accelerating increase in the number of clinically useful molecular diagnostics and targeted therapies, personalized medicine is ready for implementation. The widespread adoption of next-generation sequencing, in particular, would accelerate progress dramatically.

But although arguments for health economic benefits have a solid foundation in avoidance of costly but ineffective therapies as well as other gains in efficiency of care, payers and providers — the stakeholders that control access to personalized medicine — have been reluctant to change policies and practices without convincing evidence of the clinical and economic value of next-generation sequencing technologies.

It is not clear exactly what evidence is necessary, or how to develop and disseminate it so that it is convincing to these vital stakeholders. Most literature on next-generation sequencing has focused on the clinical utility of the approach. Thus, there is limited published literature to demonstrate, characterize or quantify its economic impact in the context of specific tumors and clinical settings.

To address this challenge, PMC has developed a research study to help demonstrate to developers, payers and providers that sophisticated molecular diagnostics are both clinically useful and economically efficient. The study, conducted by a health economics research team at the Fred Hutchinson Cancer Research Center, will involve the development of a value model validated with existing real-world data on next-generation sequencing in non-small cell lung cancer. Lung cancer was chosen as the subject of the first phase of the study because that is where the most clinical practice and economic data currently exist.

The research will utilize existing data sets — and ongoing studies to the extent possible — supplemented with a collection of data from health system and medical records. A payer advisory committee has also been formed to provide guidance to ensure that the project's results will be useful in informing the coverage and payment process.

A second phase will include an expansion of the value assessment in other major carcinomas for which mutational analysis is prevalent and practice-based data are available, such as colorectal cancer and melanoma. This second stage of research will also involve a value of information analysis and the identification of factors that significantly impact the cost-effectiveness of next-generation sequencing.

The results of the study will be published and disseminated through a robust communications plan to increase awareness and become a resource for government, payers and providers as they consider policies related to patient access to personalized medicine.

[P]ayers and providers — the stakeholders that control access to personalized medicine — have been reluctant to change policies and practices without convincing evidence of the clinical and economic value of next-generation sequencing technologies.

PERSONALIZED MEDICINE

ISSN: 1741-0541
Frequency per year: 6

Future Medicine an imprint of fsg

IMPACT FACTOR:
1.00 (2015)

PERSONALIZED MEDICINE COVERS KEY AREAS SUCH AS:

- Advances in molecular diagnostics
- Cost–benefit issues for precision medicine
- Impact of precision medicine on healthcare and the pharmaceutical industry
- Impact of the latest concepts in the development of precision medicine based on pharmacogenomics, pharmacogenetics and pharmacoproteomics

To claim your free trial, contact us at:
trials@futuremedicine.com

www.futuremedicine.com

INDEXING

EMBASE/Excerpta Medica, Chemical Abstracts, Science Citation Index Expanded™ (SciSearch®), Current Contents®/Clinical Medicine, Journal Citation Reports/Science Edition®, Scopus

"By covering the science, the policy and business of this emerging paradigm, *Personalized Medicine* assists our understanding of where personalized medicine is heading, and should be essential reading for anyone with an interest in the subject."

– **Edward Abrahams**, Personalized Medicine Coalition, USA

SCIENCE BRIEF

Eye Disease Applications Emerge as Early Leaders in Race to Bring CRISPR to Market

by Christopher Wells, PMC Communications Director

Many scientists believe the clustered regularly interspaced short palindromic repeats (CRISPR)-Cas9 genetic engineering tool will change the future of our species by giving us, for the first time, a safe and effective way to permanently alter the genomes of human cells. Some envision new treatments that forever rid the world of diseases like cystic fibrosis, Duchenne muscular dystrophy and immune system disorders, while others warn of unknown consequences if the tool is unleashed to create "designer babies" and genetically altered "super-people."

But first up may be a cure for blindness.

So say an increasing number of researchers and industry representatives, who note that altering a few cells in the eye can have a major impact with little risk of changes elsewhere in the body. The eye is also relatively accessible, can be monitored externally and has well-established measures of function.

"The eye has a history of being an organ where a lot of new technologies are translated first," explains Vinod Ranganathan, Ph.D., Postdoctoral Fellow, Wilmer Eye Institute, Johns Hopkins University School of Medicine. "The immune system is not going around scanning and looking for foreign proteins in the same way as the rest of the body."

Following the U.S. Patent Trial and Appeal Board's announcement in February of a long-awaited ruling on an outstanding interference dispute with implications for companies focused on CRISPR applications, the biotechnology community has turned its full attention on examining the technique's potential medical applications — and eye disease pipelines are taking center stage.

Allergan, a pharmaceutical company based in Dublin, Ireland, was quick to invest $90 million for the exclusive rights to license up to five programs from Editas Medicine's eye disease pipeline after the board sided with Feng Zhang, Ph.D., of the Broad Institute in the interference proceeding. Editas, which owns the exclusive rights to the application of Zhang's work to treat disease, seeks through its leading program to develop an injectable, CRISPR-containing virus that will treat Leber congenital amaurosis (LCA), a rare hereditary eye disease that causes blindness, by systematically deleting around 1,000 DNA letters in a gene called CEP290 from the DNA of an LCA patient's ocular cells. Preliminary experiments show that removing the letters should allow the gene to function normally, and Editas President and CEO Katrine Bosley announced in 2015 that the company would sponsor a clinical trial to test the product in humans this year.

In so doing, Editas would make considerable headway in the effort to take CRISPR from promise to product.

"It feels fast, but we are going at the pace science allows," Bosley said of the effort in 2015.

Intellia Therapeutics, which was co-founded by Caribou Biosciences and is pursuing liver disease as its initial focus, also lists eye diseases on its website as an area of continued exploration.

PMC's 13th Annual Personalized Medicine Conference, scheduled for November 14–16, 2017, at Harvard Medical School, will feature a panel discussion on the latest developments in CRISPR and gene therapy.

THE 13TH ANNUAL

PERSONALIZED MEDICINE CONFERENCE

Informed Dialogue. Diverse Audience. Visible Impact.

SAVE THE DATE

NOVEMBER 14 – 16, 2017

Joseph B. Martin Conference Center • Harvard Medical School • Boston, MA

World-class industry professionals, policy experts, clinicians, payers and patient advocates convene at the Annual Personalized Medicine Conference to define and examine the science, business and policy issues facing the field. Their conclusions, which PMC publishes each year in its annual *Conference Summary*, help shape the community's agenda and guide the Personalized Medicine Coalition's work.

For more details visit:

PersonalizedMedicineCoalition.org/Conference/Home

13TH ANNUAL
PERSONALIZED
MEDICINE
CONFERENCE

OPINION IN BRIEF

Precision Oncology: Far From an Illusion

by Eric Kowack, Vice President, Program Leadership, Ignyta, and Rupal Patel, M.S., Associate Director, Clinical Research & Development, Ignyta

In a *Nature* "Perspective" published last year, Vinay Prasad, M.D., M.P.H., called precision oncology an "illusion" that "has not been shown to work." Recent results, including the story of Kevin Bente, who was successfully treated with an experimental personalized medicine, prove that contention wrong.

Bente, an active 75 year-old retired school administrator with a close extended family, was in good health until 2013, when he first experienced anemia. He was referred for a colonoscopy, which ultimately led to a diagnosis of colon cancer.

The powerful story of Kevin Bente, who is pictured here with his wife, challenges the logic of critics of personalized medicine like Vinay Prasad, M.D., M.P.H., Assistant Professor of Medicine, Oregon Health and Sciences University. Prasad argued in *Nature* last year that precision oncology is an "illusion."

Like many cancer patients, he underwent surgery to remove the tumor and completed a chemotherapy regimen in May of 2014. Five months later, Bente developed significant rib pain, which was attributed to a recurrence of his cancer, with accompanying metastatic lesions. He then received a second-line chemotherapy and a biologic regimen with irinotecan, fluorouracil and bevacizumab. These standard treatments, too, were unsuccessful.

Only when the radiological/CT evaluation indicated that the chemotherapy and biologic were not working did the oncologist perform molecular testing on one of Bente's tumors, revealing an abnormal fusion between the CCDC6 and RET genes. Gene fusions of this sort are an emerging set of oncodrivers, present in less than one percent of colon cancers.[1] In contrast to the contentions of Prasad and other critics, drugs that inhibit RET fusion proteins are proving to be successful as therapeutic agents in patients with RET fusion-positive cancers.

Bente's oncologist enrolled him in Ignyta's clinical trial for RXDX-105, an investigational vascular endothelial growth factor receptor (VEGFR)-sparing, potent RET inhibitor designed to inhibit fusions such as CCDC6-RET, in the spring of 2016. The results were extraordinary. Before starting the trial, his pain was debilitating. After four weeks of treatment with RXDX-105, however, Bente said he felt "much better" than he had in a long time. After eight weeks on treatment, the CT scans indicated significant tumor shrinkage, and Bente's carcinoembryonic antigen (CEA) level, a key marker of progress, had decreased by 50 percent. At the next tumor assessment scan, following 16 weeks on treatment, no tumors were observed. Bente had experienced a complete response to RXDX-105 treatment, and his CEA level was within normal limits.

As a result of treatment with the personalized therapy, by his own account, Bente's quality of life improved significantly and he was able to return to the activities he

In this case, precision oncology accomplished what the strongest available chemotherapy could not.

loves, which include traveling, carpentry and helping his daughter build furniture for her house. As of this writing in early 2017, he continues to do well on treatment and is "thankful to have life — and a better quality of life than a chemo regimen permitted."

Kevin Bente's story exemplifies why personalized medicine is the future. The narrative shows the potential benefit of precision oncology in appropriately selected patients where the oncogenic driver is understood and there is a targeted therapy available to specifically inhibit that driver. In this case, precision oncology accomplished what the strongest available chemotherapy could not. Kevin Bente is able to enjoy a relatively normal quality of life, especially when compared to the prospect of intravenous chemotherapy and its burdensome side effects.

This is progress, not an illusion.

---

[1]Le Rolle, A., Klempner, S., Garrett, C., Seery, T., Sanford, E., Balasubramanian, S., Ross, J., Stephens, P., Miller, V., Ali, S., & Chiu, V. (2015). Identification and characterization of RET fusions in advanced colorectal cancer. *Oncotarget,* 6(30), 28929-28937.

INTERNATIONAL BRIEF

Qatar's Precision Medicine Initiative

by Nahla Afifi, Ph.D., Acting Director, Qatar Biobank, and Said Ismail, Ph.D., Director, Qatar Genome Program

Aiming to deliver world-class health care to its residents, the peninsular Arab country of Qatar has launched several personalized health care initiatives not unlike the United States' own Precision Medicine Initiative.

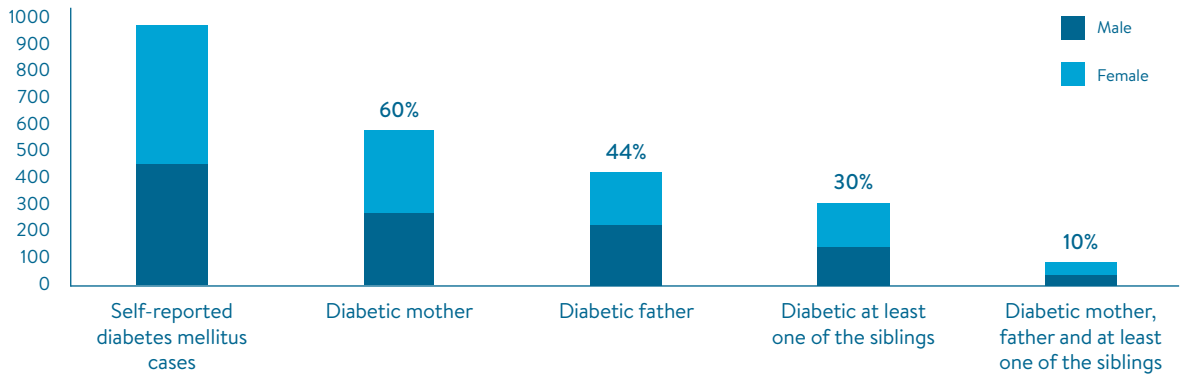
The Qatar Foundation for Education, Science and Community Development partnered with the Ministry of Public Health and the Hamad Medical Corporation to launch the Qatar Biobank (QBB) in 2011. The QBB's vision is to establish a research enterprise platform in Qatar that will facilitate an extraordinary improvement in diagnostic and prognostic intelligence for the benefit of people in Qatar and around the world. The QBB will act as the country's repository for biological samples and health information records, enabling research toward the discovery and development of new health care interventions.

A large, long-term medical research initiative, the QBB aims to recruit 60,000 Qatari nationals and long-term residents (>15 years' residence) 18 or older over the next few years. Researchers plan to follow these individuals over long periods of time, recording subsequent health conditions. Participants submit data in five stages: 1) reception and registration, 2) questionnaire, 3) measurements, 4) fitness and 5) imaging. After registering and completing a consent procedure, they answer a series of questions about their lifestyle and family, their dietary habits, their medical history, and the medicines they are currently taking.

The QBB has now registered more than 7,000 participants and has stored more than 500,000 biological samples. The data have revealed key population characteristics of Qatari residents, and preliminary results show that QBB

More Than 60 Percent of Research Participants With Diabetes Mellitus Report a Family History of the Disease

Early data from the Qatar Biobank suggest a clear genetic susceptibility to diabetes mellitus among Qatari residents.



participants are relatively young (with a mean age of 39 years) and highly educated (93 percent university educational level). The data also indicate that 87 percent are employed and 74 percent are married. Moreover, QBB data have shown that the major health defects of the Qatari population are metabolic disorders like obesity (44 percent), diabetes mellitus (DM) (15 percent) and dyslipidemia (44 percent), respiratory diseases like asthma (16 percent) or osteopenia (66 percent), and vitamin D deficiency (86 percent). But what is perhaps most interesting is the fact that preliminary data suggest a clear genetic susceptibility to DM in the Qatari population. As presented, 60 percent of respondents who self-reported as being diagnosed with DM have at least one affected family member (father, mother or siblings).

To further enable research toward the discovery and development of new health care interventions, QBB is also working closely with the Qatar Genome Program (QGP). The QGP was launched in 2013 by Her Highness Sheikha Moza bint Nasser, Chairperson, Qatar Foundation. With the support of the Qatar Ministry of Public Health and Professor Asma Althani, Ph.D., the Vice Chairperson managed by the Qatar Genome Committee and incubated in the QBB, the QGP aims to perform a comprehensive whole genome analysis of Qatari nationals and long-term residents. The program's goal is to facilitate the integration of the genomic data with the comprehensive phenotypic data collected by the QBB and use that data to predict individuals' risks of developing chronic and rare diseases. The QBB has thus far provided 4,500 samples for whole genome sequencing to the QGP, and more than 3,000 samples have been analyzed.

The QBB is also collaborating with all the local academic institutions, providing data and specimens for both educational purposes (i.e. undergraduate, M.Sc. and Ph.D. students) and research projects. The QGP, together with the Qatar National Research Fund, is also sponsoring the Pathway Towards Personalized Medicine (PPM) award, to support, jointly with the QBB, research that aims to provide medical treatment tailored to the individual characteristics of each patient based on her/his genetic profile.

Through its efforts on the QBB and the QGP, Qatar is developing a national center for biological samples and health information that can play an essential role in improving and personalizing Qatari health care and paving the way toward precision medicine. QBB data and specimens as well as the initiative's established partnerships with local and international research entities will enable Qatar to practice evidence-based medicine and become a global leader in precision medicine.

PMC NEWS BRIEF

PMC Adds Diagnostic, Payer Representatives With Expertise in Cross-Sector Collaboration to Board of Directors

by Christopher Wells, PMC Communications Director

PMC is pleased to announce the addition of two diagnostic company CEOs and a payer representative to its Board of Directors: Brad Gray, President, CEO, NanoString Technologies; Peter Maag, Ph.D., President, CEO, CareDx; and Michael Sherman, M.D., M.B.A., M.S., Senior Vice President, Chief Medical Officer, Harvard Pilgrim Health Care.

"Gray, Maag and Sherman are leaders who each offer important and unique perspectives on how to realize personalized medicine's benefits for patients and the health system," said PMC Board Chairman Stephen L. Eck, M.D., Ph.D. "Each has demonstrated an exceptional commitment to the field and will help the Coalition achieve its goals."

The new members bring valuable expertise in cross-sector collaboration. Gray has spearheaded partnerships with a wide variety of organizations as CEO of NanoString, and previously led the development of molecular diagnostics and partnering activities at Genzyme Genetics. Maag, meanwhile, who will also serve as PMC's treasurer, has more than 20 years of experience managing both pharmaceutical and diagnostic companies. And Sherman, in his role at Harvard Pilgrim Health Care, is focused on developing collaborative programs with provider organizations to support their mutual goal of delivering the highest quality of care to their patients.

"Each of these individuals brings a diverse set of experiences that will enhance PMC's ability to develop education and advocacy programs that advance the field on behalf of innovators, scientists, patients, providers and payers," said PMC President Edward Abrahams.



From left to right: Brad Gray, President, CEO, NanoString Technologies; Peter Maag, Ph.D., President, CEO, CareDx; Michael Sherman, M.D., M.B.A., M.S., Senior Vice President, Chief Medical Officer, Harvard Pilgrim Health Care

ADVANCING CARE THROUGH GENOMICS

INDIVIDUALIZING MEDICINE 2017 CONFERENCE

SUPPORTED BY THE JACKSON FAMILY FOUNDATION

OCTOBER 9–10

DISCOVER practical applications of genomic medicine. Integrate precision medicine and transform your practice.

Expert speakers from Mayo Clinic and around the world **INSPIRE** attendees.

CONNECT with others and explore future collaboration.

DISCOVER • INSPIRE • CONNECT

individualizingmedicineconference.mayo.edu

MAYO CLINIC

MEDIA BRIEF

From the PMC News Desk

FDA Approves Marketing of Ten Direct-to-Consumer Tests From 23andMe That Provide Genetic Risk Information

PMC President Edward Abrahams praised FDA after the first approval of direct-to-consumer tests that provide genetic risk information.

"By providing consumers insight into the possible significance of the genetic propensity of these ten diseases, the decision today can help inform patients' behaviors and medical decisions," Abrahams said.

The Boston Globe (April 2017)

House Legislators Release Discussion Draft of Framework for Oversight of Laboratory-Developed Tests

On March 17, Reps. Larry Bucshon (R-IN) and Diana DeGette (D-CO) released a discussion draft of the Diagnostic Accuracy and Innovation Act, which would define a regulatory pathway for the laboratory-developed tests (LDTs) that help make personalized medicine possible. The legislators say the proposed framework balances the need for regulation with the need to foster innovation.

"Our discussion draft builds upon previous efforts to establish a modern framework for the regulation of diagnostic tests, which will benefit patients and advance precision medicine," they said in a joint statement. *GenomeWeb* (March 2017)

Sen. Markey Touts Importance of Research in Personalized Medicine, Other Areas During Briefing in U.S. Senate's Kennedy Caucus Room

Sen. Edward J. Markey (D-MA) touted the importance of research in personalized medicine and other areas during PMC's briefing titled *Personalized Medicine: Opportunities and Public Policy Challenges*, which took place in the U.S. Senate's Kennedy Caucus Room on March 7. Markey said the benefits of biomedical research, which in the field of personalized medicine include improved health and decreased costs, should be considered before funding for research is withheld.

"If they have massive tax cuts plus a massive defense spending increase plus a massive wall [on the U.S.-Mexico border] — and they continue to say they want to balance the budget — well, something has to go," Markey explained to *Bloomberg BNA* after his remarks. "You don't have to be a genius detective to figure out what's left." *Bloomberg BNA* (March 2017)

Report Documents Steady Growth of Personalized Medicine, Ongoing Challenges in Regulation, Reimbursement

STAT News noted personalized medicine's steady growth after PMC launched *The Personalized Medicine Report: Opportunity, Challenges, and the Future* on Capitol Hill in March. *STAT* highlighted, in particular, the 62 percent increase since 2012 in the number of personalized medicines on the market and the fact that only 11 percent of patients say their doctor has discussed personalized medicine treatment options with them. Enhanced clinical adoption of personalized medicine, the piece suggests, could improve patient outcomes.

"The hope — and challenge — is that tools like diagnostic screening will reduce the level of trial and error in prescribing medicines by tailoring a treatment to a patient, and in the process, also reduce the adverse events that can trouble patients for whom the drug is working," the article reads. *STAT News* (March 2017)

PMC Praises Timely Approval of Personalized Drugs Amid Administration's Call for Streamlined Processes

The Washington Post made mention of PMC President Edward Abrahams' analysis of the speed at which FDA approves personalized drugs following the Trump administration's calls for streamlined processes at the agency in March. Abrahams pointed out that an estimated 90 percent of personalized medicines are approved using one of the agency's expedited programs.

"PMC appreciates that FDA has taken proactive steps to ensure that personalized medicines, which offer new hope for patients that are in dire need, are approved in a timely manner," Abrahams said.

The Washington Post (March 2017)

Analysis Shows 27 Percent of FDA's 2016 New Drug Approvals are Personalized Medicines

In an exploration of the regulatory dialogue at the Precision Medicine World Conference, *GenomeWeb* cited an analysis from PMC showing that 27 percent of FDA's 2016 new drug approvals are personalized medicines. The article notes that personalized therapies accounted for one of every four drugs FDA approved between 2014 and 2016.

GenomeWeb (February 2017)

Director of FDA's Drug Evaluation Center Defends Approval of Personalized Medicine Based on Data From 12 Patients

The Boston Globe caught up with Janet Woodcock, M.D., Director, Center for Drug Evaluation and Research, FDA, at PMC's 12th Annual Personalized Medicine Conference in November to discuss the approval of Exondys 51, a personalized medicine, based on data from just 12 patients. Woodcock said each patient population's values and circumstances are taken into account when the agency reviews drug candidates.

"We've been talking to patients a lot at the FDA over the last couple years," Woodcock said. "They're really experts in their diseases. They live with it every day. Their perceptions are actually different from the medical models [built by drug developers]."

The Boston Globe (November 2016)

PMC MEMBERSHIP

APRIL 2017

CLINICAL LABORATORY TESTING SERVICES

AlphaGenomix Laboratories
Clinical Reference Laboratory, Inc.
Empire Genomics
Laboratory Corporation of America (LabCorp)
Metabolon, Inc.
Proove Biosciences
Quest Diagnostics

DIAGNOSTIC COMPANIES

Abbott
Agendia NV
Alacris Theranostics GmbH
Almac Diagnostics
AltheaDX
ASURAGEN, Inc.
BD (Becton Dickinson & Company)
Biological Dynamics, Inc.
Caprion Proteomics
CareDx, Inc.
Caris Life Sciences
Celcuity, LLC
Exosome Diagnostics
Foundation Medicine, Inc.
GeneCentric Diagnostics
Genomic Health, Inc.
Guardant Health
Inivata
Interleukin Genetics, Inc.
Invivoscribe Technologies Inc.
Luminex Corporation
Metamark Genetics, Inc.
MolecularMD
NanoString Technologies
NovellusDx
OmniSeq
Personal Genome Diagnostics (PGDx)
QIAGEN, Inc.
RIKEN GENESIS
Roche Diagnostics Corporation
Siemens Healthcare Diagnostics, Inc.
SomaLogic, Inc.

EMERGING BIOTECH/ PHARMACEUTICAL COMPANIES

Altan Pharma, Ltd.
AveXis
Ignyta
Neon Therapeutics
Syros Pharmaceuticals
Unum Therapeutics
Zinfandel Pharmaceuticals, Inc.

HEALTH INSURANCE COMPANIES

Blue Cross Blue Shield Association
Harvard Pilgrim Health Care

INDUSTRY/TRADE ASSOCIATIONS

American Clinical Laboratory Association (ACLA)
BIO (Biotechnology Innovation Organization)
Biocom
PhRMA

IT/INFORMATICS COMPANIES

5AM Solutions, Inc.
Change Healthcare
Concert Genetics
Cytolon AG
Flatiron Health
Genospace
GNS Healthcare
Inspire
Intel Corporation
M2Gen
McKesson
Oracle Health Sciences
Seven Bridges
Syapse
UNIConnect, LC
XIFIN, Inc.

LARGE BIOTECH/ PHARMACEUTICAL COMPANIES

Amgen, Inc.
Astellas Pharma Global Development
AstraZeneca Pharmaceuticals
Boehringer-Ingelheim Pharmaceuticals, Inc.
Bristol-Myers Squibb
Eli Lilly and Company
EMD Serono
Endo Health Solutions
Genentech, Inc.
GlaxoSmithKline, PLC
Johnson & Johnson
Merck & Co.
Novartis
Pfizer, Inc.
Takeda
Teva

PATIENT ADVOCACY GROUPS

Accelerated Cure Project for Multiple Sclerosis
Alliance for Aging Research
Bonnie J. Addario Lung Cancer Foundation
Bulgarian Association for Personalized Medicine
Friends of Cancer Research
Global Liver Institute
International Cancer Advocacy Network ("ICAN")
LUNGevity Foundation
Melanoma Research Alliance Foundation
Multiple Myeloma Research Foundation
National Alliance for Hispanic Health
National Patient Advocate Foundation

PERSONALIZED MEDICINE SERVICE PROVIDERS

23andMe
Cure Forward
Health Decisions
InformedDNA
Intervention Insights
KEW Group
Massive Bio
Michael J. Bauer, M.D. & Associates, Inc.
MolecularHealth
NantHealth
N-of-One, Inc.
Perthera
Tabula Rasa HealthCare, Inc.
Tempus

RESEARCH, EDUCATION & CLINICAL CARE INSTITUTIONS

American Association for Cancer Research (AACR)
American Medical Association (AMA)
Association for Molecular Pathology (AMP)
Baylor Health Care System Precision Medicine Institute
Brigham and Women's Hospital, Genomes2People Research Program
Brown University
Cancer Treatment Centers of America
The Christ Hospital
College of American Pathologists
Coriell Institute for Medical Research
CREATE Health Translational Cancer Centre, Lund University
Duke Center for Research on Personalized Health Care
Essentia Institute of Rural Health
Genome British Columbia
Genome Canada
Génome Québec
Harvard Business School
Helmholtz Zentrum München
Indiana Institute of Personalized Medicine
Inova Health System
Instituto de Salud Carlos III
Intermountain Healthcare
International Society of Personalized Medicine
The Jackson Laboratory
King Faisal Specialist Hospital and Research Centre
Knight Cancer Institute - Oregon Health & Sciences University
Manchester University School of Pharmacy
Marshfield Clinic
Mayo Clinic
MD Anderson – Institute for Personalized Cancer Therapy
Mission Health, Fullerton Genetics Center
Moffitt Cancer Center
National Foundation for Cancer Research
National Pharmaceutical Council
North Carolina Biotechnology Center
NorthShore University HealthSystem
Ontario Genomics Institute
Partners HealthCare Personalized Medicine
Poliambulatorio Euganea Medica
Precision Health Initiative at Cedars-Sinai
Precision Medicine Alliance, LLC
Qatar Biobank
The Quebec Network for Personalized Health Care
Raabe College of Pharmacy, Ohio Northern University
Roswell Park Cancer Institute
Rutgers Cancer Institute of New Jersey
Sanford Imagenetics, Sanford Health
Stanford University School of Medicine
Sutter Health
Sylvester Comprehensive Cancer Center – University of Miami
UC Davis Mouse Biology Program
University of Alabama, Birmingham
University of Florida
University of Pennsylvania Health System
University of Pittsburgh Medical Center (UPMC)
University of Rochester
University of South Florida Morsani College of Medicine
The University of Texas System
Vanderbilt University Medical Center
Virginia Commonwealth University Health System
Wake Forest Baptist Medical Center
West Cancer Center

RESEARCH TOOL COMPANIES

Cynvenio Biosystems, Inc.
DNA Genotek, Inc.
Genia Technologies
Illumina, Inc.
Thermo Fisher Scientific

STRATEGIC PARTNERS

Arnold & Porter Kaye Scholer LLP
Bethesda Group
Bioscience Valuation BSV GmbH
Bruce Quinn Associates
Cambridge Healthtech Institute
CKSA
Clarity Research & Consulting
Co-Bio Consulting, LLC
ConText
ConvergeHEALTH by Deloitte
Credit Suisse
Defined Health
EdgeTech Law LLP
Ernst & Young Global Life Sciences Center
Feinstein Kean Healthcare
Foley Hoag LLP
Foley & Lardner LLP
Genome magazine
GlobalData PLC
Goldbug Strategies, LLC
HealthFutures, LLC
Health Advances, LLC
Hogan Lovells LLP
Jared Schwartz, MD, PhD, LLC
Jane Binger, EdD
The Journal of Precision Medicine
Kinapse
L.E.K. Consulting
McDermott Will & Emery
Navigant
Nixon Peabody LLP
Personalized Medicine in Oncology™
PricewaterhouseCoopers LLP
Professional Genetic Interactions
Quorum Consulting
Slone Partners
Spectrum
Teal Lion, LLC
Verge Scientific Communications
Washington Media Group

VENTURE CAPITAL

GreyBird Ventures, LLC
Kleiner Perkins Caufield & Byers
Mohr Davidow Ventures
Third Rock Ventures, LLC

BREAKING NEWS

AT YOUR **FINGERTIPS**

Stay Informed with the latest personalized medicine headlines from the gw newsroom

GenomeWeb's newsroom delivers in-depth, real-time reporting on the biggest stories in life science and molecular diagnostics.

- DNA SEQUENCING
- MOLECULAR DIAGNOSTICS
- GENETIC TESTING
- CANCER GENOMICS
- INFECTIOUS DISEASE
- PCR

genomeweb

REGISTER **TODAY** for our FREE **Daily News** bulletin

www.genomeweb.com

"It ought to be remembered that there is nothing more difficult … than to take the lead in the introduction of a new order of things. This … arises partly … from the incredulity of men, who do not readily believe in new things until they have had a long experience of them."

—NICCOLÒ MACHIAVELLI, *THE PRINCE*

PMC's Newest Members

Bethesda Group
Blue Cross Blue Shield Association
Bruce Quinn Associates
Celcuity, LLC
GlobalData PLC
Guardant Health
Ignyta
Jane Binger, Ed.D.
King Faisal Specialist Hospital and Research Centre
MD Anderson
Merck
Neon Therapeutics
NovellusDx
OmniSeq
Sylvester Comprehensive Cancer Center, University of Miami
Takeda Pharmaceutical International Company
Tempus Labs, Inc.
The North Carolina Biotechnology Center
Unum Therapeutics
Verge Scientific Communications
West Cancer Center

MISSION: The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

PMC
PERSONALIZED MEDICINE COALITION

1710 Rhode Island Ave., NW · Suite 700 · Washington, DC 20036
202.589.1770 · pmc@personalizedmedicinecoalition.org