PMC PERSONALIZED MEDICINE COALITION

PERSONALIZED MEDICINE IN BRIEF

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Developments in Brief

2019

AUGUST 16

GenomeWeb reports concerns that FDA's recently enhanced scrutiny of genetic testing results referencing drug-gene interactions not mentioned in the labels of FDA-approved products may slow the pace at which physicians adopt evidence-based personalized medicine strategies.

AUGUST 2

Medicare officials finalize an increase to payment rates for chimeric antigen receptor T-cell therapies. Alongside their decision in April to reconsider their approach to coverage of next-generation sequencing for cancer patients, the decision incrementally improves the outlook for patient access to personalized medicine. But both decisions still fall short of the comprehensive reimbursement changes that the field's champions are calling for.

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JULY 5

The Trump administration announces plans to tie U.S. drug prices to international payment rates that industry representatives say are not enough to recoup the costs of developing treatments. The announcement raises concerns especially about the future development of personalized treatments that depend on higher list prices to recover R & D costs from smaller patient populations.

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JUNE 27

The first cost-effectiveness study of multi-gene panel sequencing in advanced non-small cell lung cancer bolsters the case for personalized medicine and underlines the need to align clinical practices with the field's principles.

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APRIL 22

A PMC-organized committee of leaders in personalized medicine releases *A Consumer's Guide to Genetic Health Testing* to answer key questions about the personalized medicine tests with the highest public profile.

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PRESIDENT'S BRIEF

Personalized Medicine's Path to Patients: Charting a Course in Education, Advocacy, and Evidence Development

by Edward Abrahams, Ph.D., PMC President

On May 10, 2019, former U.S. Food and Drug Administration commissioner Dr. Scott Gottlieb stood before an eager audience at the National Press Club to reflect on a historic tenure at FDA during which he oversaw the approval of record numbers of groundbreaking personalized treatments and spearheaded a series of regulatory precedents that are designed to help speed the commercialization of the diagnostic tools necessary to guide those therapies to the right patients. He began by describing "a remarkable period" of scientific opportunity in personalized medicine.

"With gene therapies, cell-based regenerative medicine, more targeted therapies, and the introduction of better tools for delivering therapies from digital health apps to artificial intelligence to next-generation sequencing, we're living in an age of momentous progress and rapid cycles of innovation," he said. "We have more ability to use technology to achieve sizable and secular advances in medicine than ever before."

During an address at the National Press Club on May 10, 2019, former U.S. Food and Drug Administration commissioner Dr. Scott Gottlieb reminded us of the significance of the downstream issues influencing the pace at which the scientific and technological developments underpinning personalized medicine make their way to patients in clinical settings. Dr. Gottlieb will elaborate on his thoughts on this topic at *The 15th Annual Personalized Medicine Conference at Harvard Medical School*, scheduled for November 13–14, 2019.

The rest of his remarks focused on his preferred solutions for addressing formidable systemic challenges, underlining the need to "finance these opportunities in a fashion that optimizes access to patients who most need them and doesn't discourage future investment and innovation."

Dr. Gottlieb's attention to these downstream issues reminds us of our goals for personalized medicine, through which we seek to facilitate a permanent shift away from treatment protocols based on what has been proven to work for the highest percentage of all patients with a given disease in favor of an approach that seeks to understand everything that can be learned about each patient before prescribing the therapy that can deliver the longest-lasting effect in accordance with each patient's biology and desires. To accomplish personalized medicine's ambitious purpose, we need to align on forward-thinking policies and processes in the public and private sectors that encourage continued innovation, facilitate sustainable access to the products and services that underpin this new era of personalized health care, and prompt providers to practice medicine differently.

This is the raison d'être of the Personalized Medicine Coalition.

As the following pages demonstrate, PMC is advancing key priorities in education, advocacy, and evidence development in pursuit of a new medical paradigm that improves on one-size-fits-all medicine. Guided by its members, the Coalition has begun to lay the foundation for an enhanced impact by:

- Working with lawmakers from both sides of the aisle to establish a Congressional Personalized Medicine Caucus that will protect the future of the field;

"To accomplish personalized medicine's ambitious purpose, we need to align on forward-thinking policies and processes in the public and private sectors that encourage continued innovation, facilitate sustainable access to the products and services that underpin this new era of personalized health care, and prompt providers to practice medicine differently."

- Establishing a new PMC Education Council that will focus on developing and disseminating key messages about personalized medicine and its benefits; and
- Commissioning a series of studies that will help us better understand and affect the pace at which personalized medicine products and services are integrated into clinical work streams.

With these initiatives underway, we will reconvene in November to consider the science, business, and policy issues facing personalized medicine during the *Annual Personalized Medicine Conference at Harvard Medical School*, which will feature a keynote address by Dr. Gottlieb as well as fireside chats with Dr. Carl June, Richard W. Vague Professor in Immunotherapy, University of Pennsylvania/Dr. Stephen L. Eck, Chief Medical Officer, Immatics US, and Dr. Paul Stoffels, Vice Chairman, Executive Committee, Chief Scientific Officer, Johnson & Johnson/*CNBC* Reporter Meg Tirrell. Tackling increasingly sophisticated diagnostics on the first day and more targeted and impactful treatments on the second, *The 15th Annual Personalized Medicine Conference: The Paradigm Evolves* will systematically preview the potential of these emerging solutions before considering how each of them challenges us to think differently about the constructs of our health systems.

We hope to see you there.

PUBLIC POLICY BRIEF

Advocates Seek Congressional Caucus to Shape Policies That Will Sustain Progress in Personalized Medicine

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

Prompted by signals from officials at multiple levels of the U.S. federal government who have recently embraced public policy positions that threaten to nudge the health system away from personalized health care, the field's champions have coalesced on the need for a Congressional Personalized Medicine Caucus that can serve as a vehicle for ensuring that the health care system can support the continued advancement of the field.

Federal proposals to reduce health care costs by lowering the list prices of drugs, without considering that many personalized medicines are explicitly designed to translate higher up-front treatment costs into improved patient outcomes, would have unforeseen consequences. If these proposals are implemented, they could cause the pharmaceutical industry to move resources away from personalized therapies that would rely on higher list prices to recoup research and development costs from smaller patient populations in favor of one-size-fits-all daily maintenance medications that can be prescribed to larger patient populations at a lower price. As PMC President Edward Abrahams explains in a recent op-ed published in *The Boston Globe*'s health care-focused affiliate publication, *STAT News*, the Trump administration's recent proposal to tie U.S. government payment rates for drugs to the lowest rate paid by any country in the world, for example, would have "disastrous" implications for patients who are counting on cell-based therapies, gene therapies, and targeted medicines to address the root causes of diseases whose genetic underpinnings are increasingly understood and may therefore be targeted by a future personalized therapy.

Of course, these patients are also resting their hopes for new treatments on continued progress in biomedical research. And on this front, too, the future is still uncertain.

Democratic leaders, the Trump administration, and other members of Congress remain deeply entrenched in their respective parties' positions about how the nation should allocate its finite governmental resources, frustrating efforts to increase the budget for biomedical research spending in 2020 to keep pace with emerging opportunities in personalized medicine. As PMC emphasized in testimonies submitted to the relevant House and Senate committees to request an increase of 6.4 percent in the National Institutes of Health's 2020 budget, the increase would provide a foundation for continued medical progress at a moment when new developments are "bringing us closer to a future in which every patient benefits from an individualized approach to health care."

For their part, officials at the Centers for Medicare and Medicaid Services have made a good faith effort in recent months to respond to feedback about the agency's approach to coverage and payment for key personalized medicine products and services.

But challenges persist.

After advocates expressed concern that CMS' approach to implementing a coverage policy for next-generation sequencing (NGS)-based tests for Medicare beneficiaries with advanced cancer may prevent patients from accessing tests to guide earlier treatment and prevention strategies for hereditary cancers, the agency agreed to reconsider the scope of its coverage policy.

And in response to concerns about the additional costs associated with managing the side effects of chimeric antigen receptor (CAR) T-cell therapies, a key group of personalized treatments that re-engineer a patient's own immune cells to treat cancer, CMS has finalized an incremental increase to its payment rates for CAR T-cell therapies to help ensure that hospitals can continue to administer them.

But even the revised CAR T-cell payment rate established by the agency, which is under increasing pressure to contain costs, is unlikely to completely offset the losses many

"Prompted by signals from officials at multiple levels of the U.S. federal government who have recently embraced public policy positions that threaten to nudge the health system away from personalized health care, the field's champions have coalesced on the need for a Congressional Personalized Medicine Caucus that can serve as a vehicle for ensuring that the health care system can support the continued advancement of the field."

hospitals are facing. This obstacle exemplifies the practical challenges and bureaucratic mandates that are slowing the agency's progress toward adopting the reimbursement models that incentivize health care providers to make up-front investments in fully understanding each patient's biology and values before prescribing therapies. As PMC noted in its most recent letter to CMS Administrator Seema Verma, the landscape for reimbursement begs for "the creation of a long-term solution for adequate payment of these and other highly specialized medicines."

Under the leadership of Administrator Seema Verma, the U.S. Centers for Medicare and Medicaid Services has been quick to adjust its approach to coverage and payment of specific personalized medicine products and services in response to community feedback. Still, advocates say that the agency's best efforts to advance favorable coverage policies are falling short of the comprehensive changes necessary to facilitate payment that reflects the value of personalized medicine — and Congressional cost-containment proposals and budgetary priorities threaten to chill progress in the field.

Fortunately, PMC is making progress toward a Congressional Caucus to help solve these systemic issues, largely thanks to warm welcomes from the offices of Democratic and Republican members in the House and Senate who are eager to advance the dialogue on how personalized medicine can solve some of health care's greatest challenges.

And FDA, bolstered by the *21st Century Cures Act* and the *FDA Reauthorization Act of 2017*, continues to lead the way toward patient-centered care informed by real-world evidence.

FDA has developed a list of priorities that will help it align regulatory decisions with patient preferences, and has recently signed agreements with Cota Healthcare and Flatiron Health that will enhance its ability to learn from data collected outside the context of clinical trials, to include information from electronic health records, insurance claims, and mobile devices. During a meeting with the members of PMC's public and science policy committees in June, Dr. Leonard Sacks, Associate Director for Clinical Methodology in FDA's Center for Drug Evaluation and Research, reiterated the agency's intent to advance policies pertaining to the use of real-world evidence, which may help the developers of personalized medicine products and services more efficiently demonstrate the safety and efficacy of their solutions.

Recent and ongoing FDA approvals of one-time treatments remind us that personalized medicine has the potential to transform care for patients with a wide variety of serious diseases. These persistent public policy obstacles underline that scientific and technological developments are only the first step on the journey toward realizing the promise of personalized medicine.

PUBLIC AFFAIRS BRIEF

Amid Calls for Health Care Reform, Personalized Medicine's Champions Organize to Advance Key Messages About Field

by Christopher J. Wells, PMC Vice President, Public Affairs

Amid public discussions about how to reform the U.S. health care system that are still too often rooted in the assumption that medical decisions will forever ignore new tools that allow us to treat patients based on the unique molecular characteristics expressed by their healthy and diseased cells, proponents of personalized medicine have begun to organize a council of leaders dedicated to advancing key messages about the approach's benefits for patients and health systems.

Members of the PMC Education Council, which the Coalition will formally launch next year with participation open to anyone employed by an active PMC member institution, will seek to counter a series of emerging educational challenges that could otherwise slow the pace at which scientific and technological developments underpinning personalized medicine reach patients in clinical settings.

These challenges include a sweeping lack of awareness on the part of patients who may otherwise demand access to personalized medicine products and services. According to PMC's most recent survey on public awareness of personalized medicine, two-thirds of Americans have never even heard the terms "personalized medicine" or "precision medicine." And only 13 percent of patients say they are "very informed" about it.

This educational void persists despite the fact that PMC defined 42 percent of the new therapies FDA approved last year as personalized treatments. The disconnect encourages media dialogue about how to structure the future of U.S. health care without reference to the science that will shape it. And this dialogue, in turn, galvanizes public support for policies that may unintentionally prevent patients from accessing promising personalized treatments.

In a recent editorial on drug pricing, for example, *The New York Times* contends that the U.S. government should adopt an approach to establishing payment rates for various therapies that is similar to the model used in Britain (see "Sound, Fury, and Prescription Drugs," July 6). The newspaper fails to recognize, however, that because the United Kingdom's National Institute for Health and Care Excellence employs a value assessment methodology that has not been structured to account for the benefits of personalized medicine, its efforts to contain costs have led to a decision to refuse coverage of Vertex Pharmaceuticals' Orkambi (lumacaftor/ivacaftor), a personalized medicine that addresses root causes of cystic fibrosis. The decision has left cystic fibrosis patients in the U.K. without access to the important drug.

The rising popularity of proposals to replicate the British system in the U.S. are lending a sense of urgency to the educational efforts of personalized medicine's champions, who favor a modernized approach that would protect patients' access to highly personalized therapies that can facilitate downstream savings by curing diseases with fewer prescriptions and ensuring that treatments are targeted to only those patients who are most likely to benefit from them.

But despite their best efforts, personalized medicine's advocates have not yet succeeded in altering the perspectives of those who are in favor of aggressive drug pricing reforms, as evidenced by the fact that the *Times* again undercut the industry's pursuit of personalized therapies on August 14, with an editorial titled "This Drug Will Save Lives, for $2 Million." Like most mainstream dailies, the *Times* continues to assert the need for price controls without explaining how they would fit into a complex set of incentives that are encouraging the pharmaceutical industry to pursue personalized medicine.

"The PMC Education Council will seek to counter a series of emerging educational challenges that could otherwise slow the pace at which scientific and technological developments underpinning personalized medicine reach patients in clinical settings."

In the meantime, ongoing critiques of various approaches to at-home genetic health testing that are exemplified by an editorial describing them as "more parlor trick than medicine" (see "Why You Should Be Careful About 23andMe's Health Test," *The New York Times,* Feb. 1), threaten to undermine and confuse the public's understanding of how genetic biomarkers may be used to guide medical decision-making, which is a hallmark of personalized medicine.

Recognizing the negative impact these messages could have especially on consumers who are not educated enough to understand the differences between genetic and genomic tests that guide the use of evidence-based, genetically targeted treatments for life-threatening diseases and the many types of tests that can help keep patients apprised about our evolving understanding of the relationship between genetics and disease, a committee of PMC members convened earlier this year to develop and publish *A Consumer's Guide to Genetic Health Testing*, which answers key questions about the field.

Summarizing the scope of the educational challenges in personalized medicine, PMC Board Member Bonnie J. Addario, Co-Founder, Chair, GO_2 Foundation for Lung Cancer, notes that due in part to a global lack of awareness of personalized medicine's benefits, "some patients around the world cannot access life-saving tests and treatments."

"We simply cannot allow this to happen," she said.

The New York Times

Opinion

Sound, Fury and Prescription Drugs

Despite talk of reform, drug prices keep climbing. Here's how to change that.

By The Editorial Board

The editorial board represents the opinions of the board, its editor and the publisher. It is separate from the newsroom and the Op-Ed section.

July 6, 2019

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With most Americans still unaware that many emerging treatment strategies are designed to translate higher up-front investments in personalized therapies into downstream savings by curing patients through fewer prescriptions and targeting treatments to only those patients who will benefit from them, prevailing media messages are galvanizing public support for aggressive efforts to curb drug prices. These policies, if they are implemented, may unintentionally prevent patients from accessing important personalized treatments.

SCIENCE POLICY BRIEF

Researchers Refine Priorities for Further Study as Landscape for Clinical Adoption of Personalized Medicine Comes into Focus

by Dr. Daryl Pritchard, PMC Senior Vice President, Science Policy

In an interview about the results of the first study of the cost-effectiveness of next-generation sequencing (NGS)-based genetic tests in comparison to single-marker genetic testing (SMGT) for patients with advanced non-small cell lung cancer (aNSCLC), lead author Dr. Lotte Steuten, who recently became the Vice President and Head of Consulting for London's Office of Health Economics, carefully parsed results showing that although this key group of personalized medicine tests is moderately cost-effective, the tests could be more valuable if physicians were using actionable results more consistently to guide treatment strategies.

"Only a small fraction [of patients with actionable mutations] actually receive that targeted treatment," Dr. Steuten said, reflecting on the PMC-commissioned study that was released in June. "That reduces the value of testing, because if you don't act on the results, then the patients don't get the benefit" (see *GenomeWeb*, "Multi-Gene Panel Testing Moderately Cost Effective for Lung Cancer Patients," July 10).

"This should be investigated," she concluded.

Dr. Steuten's remarks summarize the evolving landscape for clinical adoption of personalized medicine, where new insights and ongoing challenges are informing a robust research agenda.

As Dr. Steuten implied, PMC's "Cost-Effectiveness of Multi-Gene Panel Sequencing for Patients With Advanced Non-Small Cell Lung Cancer" study underlines the importance of aligning clinical practices with personalized medicine strategies in which physicians use diagnostic tests to identify specific biological markers that inform targeted prevention and treatment plans.

The study exposed a critical "practice gap" related to the use of NGS-based test results and treatment with more effective targeted therapies (only 65–75 percent of patients with an actionable mutation as determined by NGS-based profiling are estimated to have actually received targeted treatments).

The reason for this practice gap is not clear, but we do know that the efficient use of NGS-based profiling faces implementation challenges related to education and awareness of new biomarkers and technologies; evolving practices, policies, and processes; and recognition by payers and providers of the clinical value of testing. A firm understanding among all health care stakeholders of all the ways in which genomic profiling has clinical utility can be instrumental to overcoming many of these implementation challenges.

To this end, PMC plans to gather leading experts in the field for a roundtable discussion, *Defining the Clinical Utility of Genomic Profiling in Cancer Care*, to examine current perspectives on the use of NGS in oncology and identify all of the factors that can help determine its clinical utility. Based on these findings, PMC will develop recommendations for key personalized medicine stakeholders, including health care providers, payers, clinical guideline developers, clinical laboratories, and patients, to help inform their policies and processes and support their personalized medicine implementation efforts.

Personalized medicine perspectives, and its implementation, can vary amongst health care systems in the U.S., as

"New insights and ongoing challenges are informing a robust agenda for research about the evolving landscape for clinical adoption of personalized medicine."

some providers are regularly integrating personalized medicine into their clinical work streams while others may not be accustomed to ordering diagnostics to guide prevention strategies or therapeutic decisions at all.

To gain a better understanding of the integration landscape, PMC is working with a research team from Health Advances, LLC, on a project titled *The Integration of Personalized Medicine into U.S. Health Systems: A Landscape Analysis*. The study aims to capture a holistic picture of the clinical adoption of personalized medicine strategies and technologies within the U.S. health care system by querying provider institutions about baseline community, institutional, and service delivery details as well as practice patterns and viewpoints related to personalized medicine and its utilization. This, in turn, can help inform efforts to address the most critical outstanding integration challenges.

If the clinical and economic value of personalized medicine strategies is consistently made clear to all health care stakeholders, the field will progress more rapidly. A firm understanding of the practice gaps and implementation challenges associated with personalized medicine technologies will allow us to better recognize how these gaps affect the value proposition and develop solutions that can help the health care system realize personalized medicine's full potential. This, in turn, will lead to better access to personalized medicine technologies and the associated improvements in health outcomes.

PMC NEWS BRIEF

Coalition Enhances Capacity to Advance Personalized Medicine Worldwide With Addition of Board Members Representing Researchers, Patients, Industry

by Christopher J. Wells, PMC Vice President, Public Affairs

In a development that enhances PMC's capacity to promote the understanding and adoption of the technologies and treatments underpinning personalized medicine for the benefit of patients and health systems in the U.S. and around the world, the Coalition has added Dr. Antonio L. Andreu, Scientific Director of the European Infrastructure for Translational Medicine (EATRIS), Dr. Lori Frank, a Senior Behavioral Scientist with the RAND Corporation who sits on the Medical, Scientific, and Memory Screening Advisory Board of the Alzheimer's Foundation of America (AFA), and Mr. Mark P. Stevenson, Executive Vice President and Chief Operating Officer of Thermo Fisher Scientific, to its multi-stakeholder board of directors.

Dr. Andreu, who began his career as a researcher specializing in the genetics and genomics of rare diseases, went on to create Spain's national personalized medicine program during his tenure as Director of the Spanish national institute of health, called the Instituto de Salud Carlos III.

Dr. Frank brings a deep background in the patient advocacy community. As a member of AFA's Medical, Scientific, and Memory Screening Advisory Board, Dr. Frank offers expert insight on scientific issues surrounding Alzheimer's disease and related dementias.

Mr. Stevenson, Executive Vice President and Chief Operating Officer at Thermo Fisher, helps lead the company's global efforts to advance personalized medicine by supporting commercialization, evidence development, and advocacy to help enable delivery of molecular diagnostics and targeted therapeutics from translational research into the clinic.

"As impactful champions for personalized medicine who each represent an important part of the international health care landscape, Dr. Andreu, Dr. Frank, and Mr. Stevenson are well-positioned to help guide PMC's global education and advocacy programs," said PMC Board Chairman Dr. Stephen L. Eck, Chief Medical Officer, Immatics US.

Dr. Antonio L. Andreu

Dr. Lori Frank

Mr. Mark P. Stevenson

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MEDIA BRIEF

From the PMC News Desk

Trump Administration Prompts Concerns About Future of Personalized Medicine With Plans to Adopt Drug Pricing Policies That Are Inhibiting Patient Access to Personalized Drug in UK

The Trump administration prompted concerns about the future of personalized medicine on July 5 by announcing plans to adopt drug pricing policies that are inhibiting patient access to an important personalized medicine in the United Kingdom.

In an op-ed published on July 18 in *STAT*, PMC President Edward Abrahams pointed out that Trump's proposal to lower the price that the United States government pays for any given drug to an amount that equals the lowest rate paid by any country in the world fails to recognize that the United Kingdom's pursuit of these lower rates has prompted health care officials in the country to refuse to cover a personalized therapy that patients want and need. Instead of "blindly reducing list prices for all drugs" by "abdicating its responsibility to make decisions based on what is best for Americans," Abrahams suggests that the United States government pursue its own, modernized approach to value assessment that would examine each treatment "in the context of its value to individual patients and the downstream savings it may facilitate elsewhere."

See *STAT*: "Personalized Treatments May Be Threatened by Drug Cost-Containment Proposals" (July 2019)

As At-Home Genetic Tests Influence Perceptions of Identity, PMC President Warns Against 'Conflating DNA and Culture'

In a review of *Inheritance: A Memoir of Genealogy, Paternity, and Love*, which offers the reflections of a woman who was raised Jewish only to learn from a direct-to-consumer genetic test in her mid-50s that her genetic make-up includes genes from a diverse mixture of European nationalities, PMC President Edward Abrahams notes that the author's tumultuous emotions following this revelation demonstrate our increasingly common propensity for "conflating DNA and culture." Abrahams describes the public's willingness to embrace these tests as the final word on heritage and identity as a "dangerous" trend that could "reinforce ethnic stereotypes."

See *Moment*: "Book Review | Inheritance" (July 2019)

First Cost-Effectiveness Study of Multi-Gene Panel Sequencing in Advanced Non-Small Cell Lung Cancer Bolsters Clinical & Economic Case for Personalized Medicine, Exposes Critical Practice Gap

In a development that has bolstered the clinical and economic case for personalized medicine strategies informed by genetic and genomic testing, a team of researchers led by London Office of Health Economics Vice President and Head of Consulting Dr. Lotte Steuten published a study in June demonstrating that multi-gene panel sequencing (MGPS) is moderately cost-effective for patients with advanced non-small cell lung cancer as compared to single-gene testing. The PMC-commissioned study, which was the first to assess the cost-effectiveness of MGPS for this cohort of patients, also underlined the importance of aligning clinical practices with the principles of personalized medicine.

"Only a small fraction [of patients with actionable mutations] actually receive that targeted treatment," Dr. Steuten explained to *GenomeWeb*. "That reduces the value of testing, because if you don't act on the results, then the patients don't get the benefit."

See *Precision Medicine Institute*: "Do Multigene Panels Offer Cancer Patients, Payers More Value?"

See *GenomeWeb* (subscription content): "Multi-Gene Panel Testing Moderately Cost-Effective for Lung Cancer Patients" (July 2019)

FDA Expands Frontiers of Field With Approval of $2 Million Gene Therapy, Underlining Importance of Reimbursement Models Suitable for New Era

In a development that has expanded the frontiers of personalized medicine and reminded decision-makers of the need to develop new reimbursement models that are suitable for an era of personalized health care, FDA announced the approval in May of a "transformational" $2.125 million gene therapy from Novartis, called Zolgensma (onasemnogene abeparvovec-xioi), for the treatment of a rare genetic condition called spinal muscular atrophy.

Emphasizing its commitment to value-based payment arrangements to facilitate access to Zolgensma, Novartis said it hopes the approval will bolster the outlook for personalized medicine by nudging the health care system toward payment models that allow insurance companies to recoup payments for treatments when they do not work as expected, thereby incentivizing drug developers to target treatments to only those patients who will benefit from them.

"Our goal is to ensure broad patient access to this transformational medicine and to share value with the health care system," CEO Dr. Vasant Narasimhan said in a statement.

See *The Wall Street Journal* (subscription content): "At $2 Million, New Novartis Drug Is Priciest Ever" (May 2019)

Prompted by Advocates' Access Concerns, US Officials Raise Hopes for Improved Reimbursement Landscape in Personalized Medicine by Reconsidering Key Policies

Persuaded by advocates who emphasize the importance of patient access to the products and services underpinning personalized medicine, officials at the U.S. Centers for Medicare and Medicaid Services have improved the reimbursement landscape in the field by adjusting two key policies.

In the first of the developments, the agency agreed to revisit its approach to coverage of next-generation sequencing (NGS) for Medicare beneficiaries with advanced cancer on April 29 by re-opening a previously finalized policy for discussion. The agency announced the decision after a group of 60 institutions, including PMC, wrote a letter to the agency noting that the policy had prompted Medicare Administrative Contractors (MACs) to stop covering genomic tests that rely on NGS technology to determine whether the healthy cells of early-stage cancer patients have genetic characteristics that make them more susceptible to developing breast and ovarian cancer.

And on August 2, CMS announced a revised final coverage policy for chimeric antigen receptor (CAR) T-cell therapies, an important group of personalized treatments that re-engineer patients' own immune cells to combat cancer.

In the final policy, the agency retreated from an approach that would have facilitated access to the therapies for patients covered by Medicare only when the treatments were being prescribed to generate data for a CMS-approved registry or clinical study.

As PMC Senior Vice President for Public Policy Cynthia A. Bens explained to *The New York Times* in April for a story that the newspaper describes as "a major test case" for a federal government that seeks to contain costs while facilitating access to personalized therapies, CMS' "coverage with evidence development" policy would have unnecessarily delayed access to CAR T-cell therapies for patients who desperately need them.

See *GenomeWeb* (subscription content): "CMS Reopens National Coverage Determination for NGS Testing in Advanced Cancer" (April 2019)

See *The New York Times* (subscription content): "Medicare Aims to Expand Coverage of Cancer Care. But Is It Enough?" (April 2019)

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MD Anderson – Institute for Personalized Cancer Therapy
Mission Health, Fullerton Genetics Center
MIT Center for Precision Cancer Medicine
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North Carolina Biotechnology Center
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PMC
PERSONALIZED MEDICINE COALITION

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