Personalized Medicine’s Path to Patients

by Edward Abrahams


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.1

“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.”

The rest of his address 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.

There remains no shortage of challenges on these fronts, but proponents of personalized medicine have won incremental improvements to the reimbursement landscape in recent...
who informed the development of the policy, since they understood the new language as an expansion of CMS’ current coverage criteria.

CMS demonstrated its openness to feedback on its approach to personalized medicine by agreeing to revisit this coverage policy in May.

It has sent a similar signal with its decisions about reimbursement for chimeric antigen receptor (CAR) T-cell therapies.

CMS recognizes that hospitals administering these powerful, one-time personalized treatments must absorb costs associated with the management of their potentially life-threatening side effects, in addition to the cost of the therapy itself. With this in mind, CMS authorized an additional 50 percent “add-on” payment for these treatments in April of 2018, which it increased to 50 - 65 percent, depending on the circumstances, in June of this year.

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Although these increases fall short of the add-on payment of at least 80 percent that hospital administrators estimate would be required to ensure that they can facilitate sustainable access to CAR T-cell therapies for all patients, they represent a good faith effort to advance the field on the part of agency officials, who have also responded to access concerns by agreeing to delay finalization of CMS’ proposal to cover CAR T-cell therapies only when they are administered to patients who are participating in additional studies about their benefits.

The negotiations over these policies demonstrate the complexities associated with reimbursement in this new world of personalized medicine. These factors will likely delay an era of widespread access to personalized health care until the various sectors of the U.S. health system align on approaches to value assessment that can guide more comprehensive changes to existing reimbursement models.

In the meantime, new research about the clinical and economic utility of personalized medicine suggests that even when patients gain access to NGS-based testing that can guide more precise treatments, they do not always receive the appropriate targeted therapies.

A recent study commissioned by the Personalized Medicine Coalition and led by researchers at the Fred Hutchinson Institute for Cancer Outcomes Research, for example, showed that multi-gene panel sequencing is moderately cost-effective for patients with advanced non-small cell lung cancer but could deliver more value if patients with test results identifying actionable genetic mutations consistently received genetically guided treatments. The results of the study underline the need to align clinical practices with emerging research in personalized medicine.

As we are reminded by ongoing FDA approvals like that of Zolgensma (onasemnogene abeparvovec-xioi), cleared in May as a one-time treatment for patients with spinal muscular atrophy (SMA), personalized medicine has the potential to transform care for patients with a wide variety of diseases. Persistent challenges also remind us, however, that scientific and technological developments are only the first step on the journey toward realizing the promise of this evolving paradigm.

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References