

The Need to Eliminate Barriers to Personalized Medicine

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Tom Hall had exhausted all the chemotherapy medicines available, and his metastatic lung cancer was spreading. With little hope left, he turned to genetic testing. Based on his genomic profile, 5 off-label medicines were recommended, from which his doctor selected a Medicare Part D drug approved for renal cell carcinoma but not for Tom's lung cancer. This treatment appeared to slow the progression of his disease and gave him more time with his family.

At first glance, genetic testing and subsequent precision treatment seemed to offer hope for Tom, something often hard to come by for patients battling for their lives. However, a vital aspect of his story is missing—the part where Medicare originally denied coverage because the drug would be used off-label. In fact, his doctor appealed twice and was denied. Personalized medicine provided Tom with some new options. The process broke down, however, at a highly crucial juncture—the point at which Tom had to gain timely access to and coverage for his personalized treatment.

Tom sought help and was referred to the Patient Advocate Foundation (PAF), which has provided direct services and support over the past 19 years to more than 750,000 Americans facing chronic, debilitating, and life-threatening illnesses. Ultimately, a PAF case manager submitted an expedited appeal with documents showing the applicability of the off-label use based on his genetic profile. The appeal was successful and Tom finally obtained coverage for his new cancer treatment.

But for every Tom, there are countless Americans for whom clinically advanced genetic testing and precision treatments are simply not an option. Tom himself does not fit into the patient category for whom good healthcare should be out of reach. His family has a comfortable income, and he has full healthcare coverage.

The advantages of personalized medicine are still unattainable for many Americans—even those who are well insured and financially stable. From a patient advocacy perspective, this situation is concerning.

Precision medicine will perhaps establish some of the most important biomedical innovations of our generation. We at National Patient Advocate Foundation (NPAF), the advocacy arm of PAF, fully support wide-ranging investment in innovative, personalized medicines—especially in oncology. However, for that future to become a reality, we have to create an access and reimbursement environment that is conducive to precision care.

Impact of Personalized Medicine

We believe, and research has shown, that personalized diagnostics and medical treatments can improve outcomes by offering individually tailored treatment plans to patients based on certain genetic or other defining characteristics. Especially when it comes to cancer, the appropriate use of genetic testing and counseling will better align cancer treatment from the get-go.

Further, we know that research and healthcare will only progress when patients have access to, and participate at much higher rates in, clinical trials. Tom Hall had 5 options that might have worked for him. While he was already too weak from previous failed treatments to travel to participate in some of the trials, patients and their physicians should be well informed of all treatment options, including clinical trials.

Insurers have questioned whether some of the genetic tests being used have been validated. They request more research, and evidence that the overall concept works. We must support positive collaborations among providers, insurers, patients, drug companies, and diagnostic labs to document the success of comprehensive genomic profiling in linking patients to appropriate treatments as early as possible and without administrative hoops and excessive coinsurance. We believe such individually tailored plans can produce dramatic clinical responses in some cases, particularly in areas that have traditionally had few options, including melanoma, lung cancer, and pancreatic cancer. In doing so, personalized medicine can help achieve an incredible goal for patients: the slowing or reversal of diseases that once seemed unstoppable.

It is important to keep in mind that cures should not be considered the only success story in healthcare. As Tom Hall's family understands well, more time and better quality of life can be incredible gifts to patients and their loved ones. Our optimism grows as we hear more stories of scientific and clinical success, such as individualized cancer vaccines that induce the immune system into action or screening methods that increase the accuracy of ovarian cancer prognosis and diagnosis.

Fortunately, our lawmakers have taken notice and are responding with action. Congressional leaders are championing bipartisan proposals like 21st Century Cures to improve medical innovation, including precision medicine.¹ The federal government has now assembled a team of medical and science experts to build President Obama's Precision Medicine Initiative, which he first introduced in this year's State of the Union address.²

The big challenge increasingly facing patients is their ability to access and afford these new and innovative therapies. In order for precision medicine to truly succeed, we need to ensure ready access to appropriate diagnostic and genetic tests, coupled with easy access to optimal personalized treatment regimens. With unwavering determination, advocates throughout the country must champion clinical decision and payment models that support precision medicine.

Surmounting Existing Barriers

We have identified at least 2 significant issues that need to be addressed in order for personalized medicine to be a reality for most Americans: specialty tiers and clinical pathways.

Medicare and other payers have placed many advanced medications, including personalized treatments already on the market, on a “specialty tier,” which requires payments beyond traditional co-pay amounts. In these instances, patients, regardless of income, must pay a percentage of the drug cost, often in the range of 20% to 40% or more. These costs often stretch into the thousands for a single treatment, rendering the treatment inaccessible for some. Patients should not have to decide between potentially life-altering treatment and debilitating medical debt. In order to protect patients, we must manage patient exposure to exorbitant coinsurance costs. Thus, NPAF supports Congressional bills such as HR 1600, the Patients’ Access to Treatments Act of 2015,³ along with state legislation that limits specialty tier pricing in an attempt to keep costs reasonable for patients.

In addition to specialty tiers, patients must be aware of certain clinical pathway programs in which payers are incentivizing doctors to prescribe treatments based on a small pre-determined list that is not likely to include one of the many options suggested by a genomic profile. Many insurers already utilize a fail-first approach to certain treatment regimens, which require lower-cost medications to be prescribed and to fail before more expensive medicines are made available to patients, even when the latter are included in nationally recognized clinical guidelines.

There must be a better way. If we can match patients to a personalized treatment regimen most likely to succeed based on the unique characteristics of their disease, then we can simultaneously put many on the most direct track to good health, preempt costs associated with less effective or unnecessary treatments, and prevent future health problems. Basket trials are one such innovation that hold great promise for science and for patients and allow rapid testing and approval of novel therapies. These trials are designed to assess positive responses to a targeted therapy among a small number of patients and, in the process, validate a clinical target linked to a molecular marker, independent of tumor site.⁴

Clinical pathways, trials, and reimbursement models must be structured in a way that accommodates precision medicine by allowing physicians to pursue treatment options that hold the greatest promise for personalized treatments from the very start of a patient’s deeply personal care journey. We like to think of it as precision access or reimbursement.

As we continue to make bold scientific advances, we must keep the powerful stories of individuals like Tom Hall with us. For in the years to come, personalized medicines should be judged by the years of life they add for patients, not by the years it took for people to gain affordable access to them.

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- See more at: <http://www.ajmc.com/journals/evidence-based-oncology/2015/august-2015/the-need-to-eliminate-barriers-to-personalized-medicine#sthash.PYOO6f1K.fsvujDo3.dpuf>