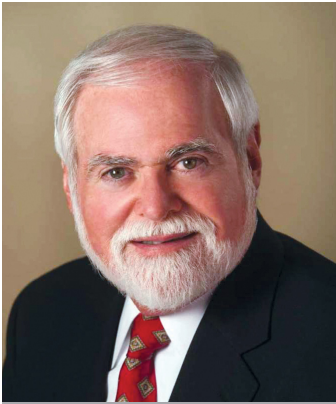


IMPROVING CANCER PATIENTS' ACCESS TO PRECISION MEDICINE

By Alan Marks, MD, and Sloka Iyengar, PhD



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Treatments for cancer have improved dramatically in recent decades. The five-year survival rate – the percentage of people with cancer who will be alive five years after diagnosis – has gone up, while death rates due to cancer

have decreased. Nevertheless, a substantial proportion of the population is still affected by cancer.¹ The conventional avenues of treatment are chemotherapy, radiation and surgery, but the last few years have seen considerable strides in an innovative method: precision medicine.

Most medical treatments are designed for the “average” patient; hence, they succeed with some patients but not with others. Precision medicine is a bold, targeted and patient-specific approach. It uses diagnostics to analyze the genetic makeup of the tumor, a process known as genomic or molecular profiling. With the results of precision diagnostics, oncologists can prescribe targeted and immunotherapy treatments that specifically address the patient’s tumor. This approach is meant to improve chances of survival and decrease side effects.

In cancer care, precision medicine includes personalized treatments that target a tumor’s genetic mutation.

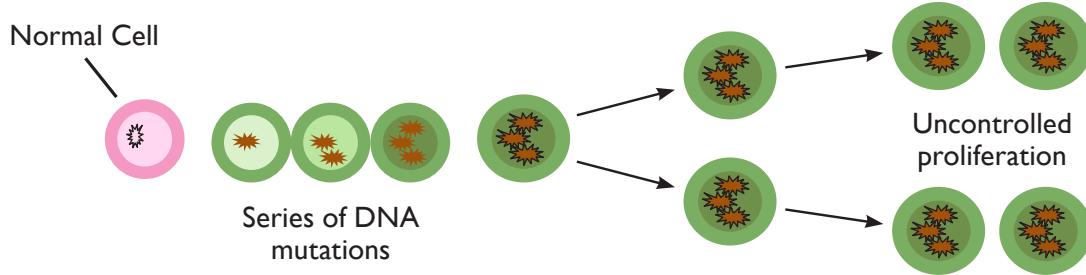
Precision diagnostics involve genomic sequencing of the tumor tissue to identify the best course of treatment.

Oncologists struggle to stay current on precision medicine because of competing professional responsibilities, and they have no practical guidelines for genomic testing and genetic counseling.

FDA has been slow to incorporate precision diagnostics into its regulatory procedures for drug approval.

Health plans often lack coverage for precision medicine because insurers want evidence of precision medicine’s “medical necessity,” which is difficult to provide at this early stage.

FIGURE 1: CANCER IS CAUSED BY GENOMIC MUTATIONS IN CELLS



Mutations due to heredity, radiation, chemicals or spontaneous errors proliferate uncontrollably, leading to cancer.

CANCER PATIENTS' ACCESS TO PRECISION MEDICINE

There is palpable excitement surrounding precision medicine's potential for treating cancer, as it provides a method to target genes in a highly specific way. However, an important consideration is whether cancer patients can actually access these technologies. Three barriers in particular can prevent access to precision medicine:

- Education and logistics challenges
- Inadequate regulation
- Lack of health plan coverage.

BARRIER #1: EDUCATION AND LOGISTICS CHALLENGES

Healthcare professionals face two challenges related to education and logistics: 1) gaps in knowledge and skill, and 2) competing professional responsibilities.

Healthcare professionals sometimes lack knowledge as to which test(s) to order, how to interpret the results, and whether high-risk family members should also be tested. A related challenge

is determining which laboratories perform testing most accurately. The timing of precision diagnostic tests is critical, but it is not yet clear when such tests should be ordered. Should the testing occur at the initial diagnosis of cancer, or at the first relapse? These knowledge gaps may cause healthcare professionals to hesitate when ordering and interpreting the results of genetic testing or when initiating genetic counseling.

Precision medicine: an innovative approach to detecting and preventing disease that factors in a patient's genetic background and environment.

Physicians also face competing clinical priorities.² Oncologists' limited time with patients already includes gathering patient history and consulting about other serious issues such as mental health and chronic pain. A physician's typical busy workday also includes ordering and interpreting lab results and other diagnostic tests and organizing consultation reports. This leaves little time to explore a complex topic such as oncology genomics.

Moreover, since precision medicine is matched molecularly to each cancer patient, it requires collaboration among basic scientists, oncologists, pathologists, geneticists and bioinformatic professionals. Such collaboration takes time and an exceptionally strong support system for the physician, which does not always exist. Without the needed support structure, education, and workflow conducive to precision medicine, physicians may find themselves unequipped to steer patients toward effective diagnostics and targeted therapies.

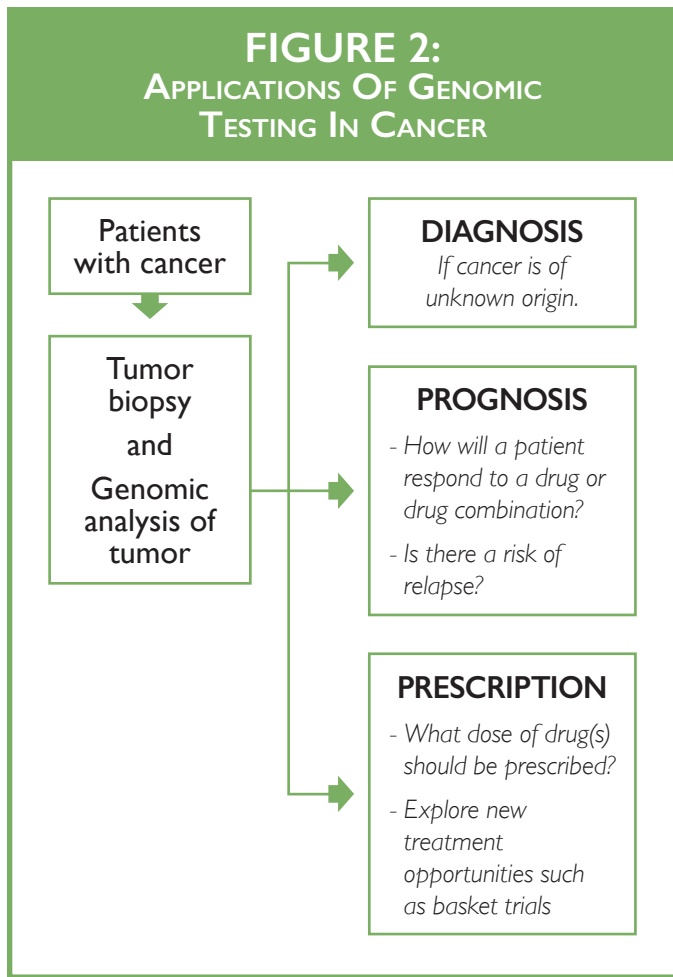
Educational programs should incorporate opportunities to practice what is being learned, incentives to drive change, small-group exercises, and evaluation of the participants and the program itself.

The second challenge, professional responsibilities competing for physicians' limited time, can be addressed by targeting the practice flow of a healthcare professional's typical workday. For example, electronic health record systems that effortlessly incorporate precision diagnostics would be useful. No simple solution exists for alleviating the burden of time-consuming collaboration, though programs that foster cooperation among oncologists, geneticists and bioinformatic professionals can help ensure that no one individual shoulders all the responsibility for practicing precision medicine.

BARRIER #2: INADEQUATE REGULATION

The explosion of knowledge and data brought about by genomic sequencing of tumor tissues brings with it unique regulatory challenges. Two regulation-related challenges that inhibit patient access to precision diagnostics are: 1) lack of adequate oversight, and 2) inadequate current clinical trial procedures.

Precision medicine presents a fundamental shift from traditional modes of diagnosis and treatment. Current diagnosis fits the “one test, one disease” paradigm, whereas precision diagnostics is more exploratory and gives rise to massive amounts of data. Furthermore, current treatments for cancer follow a “one-size-fits-all” approach, whereas precision medicine is highly specific to an individual.



Clearing the way for patient access requires addressing these challenges. The first challenge, lack of physician knowledge, can be addressed by providing healthcare professionals with education and training about precision diagnostics.

BARRIER #3: LACK OF HEALTH PLAN COVERAGE

The U.S Food and Drug Administration (FDA) has recognized the need for improved oversight, and is working to organize a regulatory system that will guide innovation in precision medicine while ensuring data accuracy and protecting patient privacy.^{3,4} This new initiative is a cloud-based platform that will enable the FDA to update its regulatory paradigm. Another goal of regulatory oversight is to come up with treatment algorithms for specific cancers. Such a formula has been proposed for certain cancers⁵ and would be beneficial for other cancers as well.

A second challenge relates to how clinical trials for oncology are structured. Most clinical trials group patients based on the location of their tumors in the body. However, data from genomic testing has revealed that the same mutation may be present in several different types of cancers. A patient with, for instance, “lung cancer genes” might respond to drugs typically used for breast cancer.

So-called “basket trials” have emerged to address the limiting nature of current clinical trials. These trials are designed based on genomics, not necessarily on the origin of the tissue.⁶ Basket trials provide an alternative to randomized controlled trials, especially for rare cancers, where recruiting patients is especially difficult.

To provide cancer patients with access to precision diagnostics, targeted therapies and immuno-oncology treatments, the existing regulations and clinical trial procedures must continue to be updated to better accommodate precision medicine.

High cost-sharing can require patients to pick between treatments that could save their lives and long-term financial stability for their families.

Reimbursement models that have not kept pace with innovations in precision medicine also pose barriers to patient access. Sick patients and overwhelmed caregivers often have to be their own advocates and navigate the treacherous path of getting reimbursed from insurance companies while dealing with arduous pre-authorization procedures. Two significant health plan coverage-related challenges are: 1) demonstrating medical necessity, and 2) handling exorbitant cost sharing.

Numerous factors determine whether a particular insurance plan covers genetic testing: consumer demand, opinions of leaders in the field and proof that a particular test or treatment has a positive effect on the patient’s outcome (“medical necessity”). For example, Medicare requires proof that precision diagnostics provide more accurate diagnosis than existing methods and an explanation of how the information gathered affects ultimate patient outcomes.⁷

Cost sharing presents additional challenges. Precision medicines already on the market are included in what’s known as a “specialty tier,” which often requires patients to pay a percentage of the treatment’s cost rather than a flat co-pay. The cost can be prohibitive, leaving patients to pick between treatments that could save their lives and long-term financial stability for their families.

Legislation such as the Patient’s Access to Treatments Act of 2015⁸ can help to curb prohibitive cost-sharing requirements. But the emergence of precision medicine likely requires a more significant paradigm shift on the part of health plans. As cancer care moves beyond strictly matching treatments to tumor locations,

health plan coverage must likewise adjust to accommodate more comprehensive diagnostics – and the treatment paths they suggest – even when the therapy choice indicated through tumor analysis is less conventional or more expensive.

CONCLUSIONS

Precision diagnostics offer unparalleled potential for the treatment of cancer, but certain barriers prevent patient access. Healthcare professionals, regulatory bodies, patient advocacy groups, insurers and other stakeholders must unite to ensure that cancer patients can benefit from these breakthrough technologies. Education programs for healthcare professionals, an updated paradigm for regulatory oversight and a restructured healthcare coverage approach will ensure that cancer patients can access precision medicine.⁹

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