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Biomarker tests for molecularly targeted therapies need better evidence, oversight

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Potentially useful biomarker tests for molecularly targeted therapies are not being adopted appropriately into clinical practice because of a lack of common evidentiary standards necessary for regulatory, reimbursement, and treatment decisions, says a new report by the National Academies of Sciences, Engineering, and Medicine. To enhance patient care and clinical outcomes, the report calls for the creation of a "rapid learning system" that would integrate research on these tests and associated treatments with clinical practice. Appropriate regulatory oversight is also needed to ensure that biomarker tests and targeted therapies are accurate, reliable, and properly validated and implemented.

Biomarker tests for molecularly targeted therapies identify molecular variations specific to an individual patient, which can help determine the most effective therapy for a patient's disease or avoid treatments that could be ineffective or harmful. Advances in research over the past 15 years have led to hundreds of molecularly targeted agents entering the drug development pipeline; numerous biomarker tests and associated therapies have been approved for clinical use in treating cancer and other diseases. However, progress has been hampered by regulatory and reimbursement uncertainties, clinical practice challenges, and limitations in data collection and analysis.

"The timely development of biomarker tests and associated therapies is critical to realizing the full potential of 'precision medicine,'" said Harold L. Moses, chair of the committee that wrote the report, and

Ingram Professor of Cancer Research, professor of medicine, pathology, microbiology, and immunology, and chair of the department of cancer biology at Vanderbilt University. "Our report lays out a strategy to ensure that patients have access to effective tests and treatments that are based on solid evidence of their ability to improve health outcomes."

The committee identified 10 goals to advance the development and appropriate clinical use of biomarker tests for molecularly targeted therapies. First and foremost, the U.S. Department of Health and Human Services should immediately facilitate the development of common evidentiary standards that demonstrate the usefulness of biomarker tests for selecting targeted therapies and improving patient outcomes, the report says. These standards would inform regulatory, insurance coverage, and reimbursement decisions, and they could also strengthen clinical guidelines and standards of care. To develop and continuously update the standards as new information becomes available, HHS will need ongoing input from a wide variety of stakeholders, including patients, health care providers, test developers, pharmaceutical companies, and relevant government agencies. HHS should also facilitate collaboration between the U.S. Food and Drug Administration and the Centers for Medicare and Medicaid Services to enable more coordinated, transparent regulatory and reimbursement decisions.

Generating evidence of the overall clinical benefit of any biomarker test should be viewed as a continuous process, the report says. CMS and private insurance companies should develop payment models to support ongoing data collection on tests and associated therapies. Currently, data on biomarker tests and patient outcomes are not collected or shared in a way that can inform clinical practice. Electronic health records and laboratory information systems should facilitate the collection of patient data in real time, including information about tests, treatments, and outcomes. A national database should be created to capture these data to help health care providers and patients make evidence-based decisions

about tests and treatment. In addition, the processes for developing and updating clinical practice guidelines for the effective use of biomarker tests for molecularly targeted therapies should be improved so that new data are incorporated and a broader base of interdisciplinary expertise is involved, the report says.

Some patients may face challenges in obtaining access to biomarker tests and therapies because of economic, ethnic, cultural, or geographic barriers. The report calls for research on how to provide equitable access to biomarker tests and targeted treatments. And to empower patients and their health care providers to make informed decisions, the FDA should develop patient- and provider-friendly standardized labels and additional information about tests' performance, evidence, and uses.

Oversight and accreditation of laboratories providing biomarker tests for molecularly targeted therapies should also be updated and strengthened, the report says. Current regulatory oversight of these labs is widely viewed as insufficient for increasingly complex biomarker tests. And professional organizations and health care institutions should develop and implement standards for obtaining patient specimens to ensure patient safety as well as the accuracy of biomarker test results.

More information: www.nap.edu/catalog/21860/biom … -unlocking-precision

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