Testimony of Dr. Diana M. Zuckerman, President, National Center for Health Research

Examining Policies to Enhance Seniors' Access to Breakthrough Medical Technologies

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Energy & Commerce Subcommittee on Health

Executive Summary

I will focus on the *Ensuring Patient Access to Critical Breakthrough Products Act of 2025* and the *Nancy Gardner Sewell Medicare Multi-Cancer Early Detection Screening Coverage Act.*

Since both of these bills require Medicare coverage for specific medical devices, I explain the differences in FDA and Medicare standards. FDA standards for medical devices are a "reasonable assurance of safety and effectiveness," while the standard for Medicare coverage is "reasonable and necessary" for Medicare patients. Should Medicare be required to pay for certain devices, regardless of whether CMS determines that they are reasonable and necessary for Medicare beneficiaries? FDA standards for medical devices are not specific to Medicare-age patients and so do not consider whether implants or other devices requiring invasive procedures have benefits that outweigh the risks for older Americans. The data provided to the FDA in device applications often includes few older patients with few co-morbidities.

Early detection of cancer is a goal we all share. However, research on multi-cancer early detection tests indicate that they result in many false positives and many false negatives. The former results in unnecessary anxiety and additional tests, whereas the latter is likely to result in patients who ignore signs and symptoms of cancer, thus delaying needed treatment. I will describe what we know and don't know about the accuracy of these tests.

Our analysis of the 160 Breakthrough medical devices that can be marketed indicates that many were not studied in controlled clinical trials and that there is often no publicly available information about how many patients in any of the studies were old enough for Medicare. When age information is publicly available, it is sometimes obvious that few if any patients were 65+. As a result, they are not proven to benefit patients eligible for Medicare. This is especially true for breakthrough devices cleared through the 510(k) pathway or the De Novo pathway.

Written Statement of Dr. Diana Zuckerman

Thank you for the opportunity to testify about these important bills today. I am Dr. Diana Zuckerman, president of the National Center for Health Research (NCHR), a nonprofit think tank that is dedicated to improving the quality of medical care in the United States. We do that by using research information to inform policies, programs, and services.

Prior to my 26 years as the founding president of NCHR, I was a faculty member at Vassar and Yale, a research director at Harvard, a Committee staffer in the House and Senate, and an Assistant to the President in the White House. I have also been a Bioethics Fellow at the University of Pennsylvania, and a founding Board member of the Reagan Udall Foundation and the Alliance for a Stronger FDA.

I will focus on two bills that you are considering, the *Ensuring Patient Access to Critical Breakthrough Products Act of 2025* and the *Nancy Gardner Sewell Medicare Multi-Cancer Early Detection Screening Coverage Act.*

Why aren't all medical devices on the market proven safe and effective?

Since both of these bills require coverage for specific medical devices, I will start with brief information that will put my remarks in context.

The FDA is known as having the gold standard for ensuring the safety and effectiveness of medical products, but those standards differ substantially for devices compared to prescription drugs. The standards for medical devices are "**a reasonable assurance of safety and effectiveness**" and that is not exactly the same as "safe and effective." I will provide information about specific devices to see how those criteria are met.

Prescription drugs are approved based on scientific evidence that is almost always based on clinical trials – studies of patients with the disease or illness that the drug is intended to treat. Almost all these drugs and biologics are compared to a placebo control group, because as most of you know, patients sometimes do well with a treatment that they believe is effective, even if it is just a placebo pill with no beneficial ingredients. In contrast, less than 5% of medical devices regulated by the FDA are tested in clinical trials. Approximately 95% go through an easier pathway that is called the 510(k) process and is based on the company's ability to convince the FDA that their device is "substantially equivalent" to another medical device that is already on the market. There is no requirement that the device it is substantially equivalent has been proven safe and effective in a clinical trial, and in fact, usually they are not.

Medicare coverage for devices

Medicare almost always pays for prescription drugs that are approved by the FDA for a particular use by adults, whether it is for heart disease, cancer, diabetes, or almost any disease or condition. It does not automatically pay for all medical devices, because it is understood that the standards are lower to get medical devices on the market. It is well documented that most medical devices do not need to have clear scientific evidence that they are proven safe or effective, and often there is no public information about any evidence that they are safe or effective. [1] When I served two terms on CMS' MEDCAC Committee, which recommends

whether products should be covered by Medicare, I was surprised at how often devices were not studied on people over 65, and sometimes not even over 55 or 60. For that reason, many advisors voted that Medicare should not cover them.

Medicare Standards vs. FDA Standards

Both these bills would circumvent CMS decision-making about what Medicare will pay for. A major question before you today is whether Medicare should be required to pay for certain devices, regardless of whether CMS determines that they are reasonable and necessary for Medicare beneficiaries. Most health policy experts who care about the health of Medicare patients do not think automatic coverage is a good idea, because they know that FDA's standards are not always consistent with the needs of Medicare patients. A major reason is that FDA standards for medical devices are not specific to Medicare-age patients and so do not consider whether implants or other devices requiring invasive procedures have benefits that outweigh the risks for older Americans. For example, Medicare patients tend to have more co-morbidities that make surgery riskier and they also may respond differently to anesthesia and other drugs used for surgical patients. Clinical trials often intentionally avoid including patients who have illnesses unrelated to the treatment being studied, since they can influence outcomes. As a result, the data provided to the FDA in device applications usually includes relatively few older patients, or only the healthiest patients relevant to the study device.

Nancy Gardner Sewell Medicare Multi-Cancer Early Detection Screening Coverage Act

Early detection of cancer is a goal we all share. As a cancer survivor, I appreciate the goal of this legislation. For many cancers, early detection can make the difference between surviving cancer or not. A screening test that is simple is likely to be used by more people than one that is unpleasant, but to have benefits that outweigh the risks the screening test needs to be

as accurate as possible, so that patients can rely on them. A test with many false positives, where most patients who are told they may have cancer do not have cancer, creates great anxiety and results in additional testing that may be painful, harmful to their health, extremely expensive, time-consuming, and cause additional stress and anxiety. A test with many false negatives, in which patients are told they do not have cancer when they actually do, is likely to result in patients who ignore signs and symptoms of cancer, thus delaying needed treatment.

Multi-cancer early detection tests are a very promising development in cancer screening, but they are not ready for prime time. An article published in the *Annals of Internal Medicine* this week reviewed all relevant research and reported that existing tests are subject to bias but even so they miss most early cancers in people who do not have symptoms and may provide false positives to most patients.[2] In one of the tests cited, a person with a test result indicating cancer was correct only 4% of the time. Since inaccurate results are so harmful, research is needed to determine how accurate the current tests are, whether some are more accurate than others, and whether they identify early cancers in asymptomatic adults. In July of this year, the National Cancer Institute (NCI) launched the Vanguard Study of these kinds of tests. NCI considers this a preliminary study, even though it will include 24,000 people ages 45-75. NCI plans to use the information from this study to conduct an even larger study. Major goals of this study and similar research are to determine how accurate these tests are and whether any of the tests save lives. Although individual patients report that their test results are the reasons they are alive today, some experts question whether the tests are more beneficial than other types of screening that more accurately identify cancer. As stated in the *Annals of Internal Medicine*, it is unclear whether MCED tests "detect cancer types at later, untreatable stages or whether they detect very early-stage precancerous lesions that might never have developed into cancer."

These tests are medical devices, and FDA standards for these products follow a "least burdensome" approach to determining a "reasonable assurance of safety and effectiveness" for marketing authorization. While this approach does not change the standard for approval, it does change the evidence required in a way that is considerably lower than for prescription drugs. In addition, the bill specifies that Medicare would be required to pay for any test that is cleared or approved by the FDA, which means it includes a test cleared by the very low standards of the 510(k) pathway, which rather than human testing is almost always based only on evidence of substantial equivalence to an already marketed device. That seems problematic when there are no marketed devices that are proven beneficial.

Medicare saves lives every day. A bill that requires Medicare coverage for specific types of devices should not be considered until there is clear evidence that the benefits of those tests outweigh the risks for Medicare patients. Studies are underway to answer that question, but at this point, we are not even close to having that evidence. I agree with the many oncologists who consider this bill to be premature at this point, because of all the unanswered questions about the benefits of these multi-cancer tests.

If in the future these tests are proven to have proven benefits that outweigh the risks for Medicare patients, then I would urge that the age restrictions be deleted. As written, the age restrictions set a dangerous precedent for Medicare coverage decisions and would cause an uproar among patients who are excluded from coverage for reasons that are not specified and will be perceived as unfair.

Breakthrough Devices: Myth and Reality

FDA's criteria for breakthrough medical devices require them to be more effective than alternatives for diseases or conditions that can be life-threatening and irreversibly debilitating.

Each device selected must also meet at least one of 4 other criteria: representing a breakthrough technology; no FDA-approved or cleared alternatives; having significant advantages over other available options, or availability is in the best interest of patients.

FDA designated 1,176 breakthrough devices through June 30, 2025, based on preliminary information that the devices could meet the required criteria, after which the devices had to provide evidence that they should be allowed on the market. To get on the market, they did not have to prove that they satisfied the criteria for being a breakthrough device.

Of the 1,176 breakthrough devices, 160 have been cleared for market. Our analysis indicates that many of these would not satisfy the criteria of being more effective or otherwise superior to other treatments. In other words, they were designated as breakthrough before data were available, and afterwards they retained that PR term of being breakthrough, whether they were as good or better than devices designed for the same type of treatment, or not.

Are Breakthrough Devices Beneficial for Medicare Patients?

We studied those 160 devices to see how beneficial they are likely to be for Medicare patients, since that is the goal of the legislation. Of those 160, 112 are therapeutic devices to treat a disease or condition and the others are diagnostic tests. I will focus today on the 510(k) Breakthrough treatment devices.

510(k) Breakthrough Devices

The 510(k) devices are described by the FDA as moderate risk devices that are similar to other devices on the market. Since life-saving devices can cause terrible harm if they are not safe or effective, most of us would consider them high risk. You might wonder why a device that is not life-saving and is similar to other devices on the market should be considered a

"breakthrough" device, and that is a very good question. Here is some information that will help you decide if you think that Medicare should be required to pay for those devices.

One of those 510(k) breakthrough devices is for infants and toddlers only, so there are 49 breakthrough 510(k) devices of concern to us today. Only 8 of the 49 devices to treat adults (16%) were in clinical trials that were listed in ClinicalTrials.gov. Two other devices seemed to have clinical trials but no trials were listed for those devices in clinicaltrials.gov. Listing in clinicaltrials.gov is required by FDA law, with the exception of very preliminary studies, so it would make sense to require that any breakthrough devices covered in this bill should have a clinical trial listed in clinicaltrials.gov.

Of those 8 devices, one intentionally excluded any patients over 63 years of age from the clinical trials. That leaves only 7 (14%) that satisfy the criteria of having a clinical trial that might have been studied on patients ages 65+. There is publicly available info on only one of these devices that clearly states it includes patients 65+, but it doesn't specify if there are enough patients over the age or 65 to determine if the device is safe or effective for them. One device studied patients with a mean age of 36.6 and a different device had a median age of 37, so it is unlikely that either included many people over 65. That means that only 5 (10%) or fewer of the 50 Breakthrough 510(k) devices would qualify as reasonable and necessary for Medicare coverage.

In addition to the 8 devices I just described, there are 17 breakthrough 510(k) devices that have some kind of clinical data that was not a clinical trial, that are based on both the predicate device and the breakthrough device. That's 35% of the total breakthrough treatment devices for adults. In addition, there are 10 more devices that provided nonclinical data (such as animal data or mechanical data) that are also related to their predicate. In total, that means that 55%

(27/49 breakthrough 510(k) devices) provide information based partly or primarily on the predicate, not the new device.

Breakthrough Devices Based on Pre-Market Approval (PMA) and De Novo Reviews

Although the 510(k) pathway breakthrough devices are of particular concern, the breakthrough devices currently on the market based on the more stringent PMA and De Novo pathway also have data problems that are relevant to this legislation.

Most of the 20 De Novo breakthrough devices provide no information about the age of the patients in their studies, making it impossible to know if the devices are safe or effective for patients over 65. The few that specify the age of patients over 65 have fewer than 10. In addition, many of those studies are small (some including only 10 patients, for example, and have no control group.

The 35 PMA breakthrough devices for adults were studied on larger numbers of patients, but the number of patients aged 65+ was rarely specified. For the 19 devices with a mean age in the 60's, we assumed that there were patients over 65 but could not determine how many. In contrast, one device was studied on adults with a mean age in the 20's, one with a mean age in the 40's, and five with a mean age between 50-55, so for those devices it was likely that there were few if any patients 65+.

Here are 2 examples of 510(k) breakthrough devices that are on the market. The first is for spinal fusion, a high-risk procedure. The second is for an insulin delivery system for diabetes patients.

Carlsmed aprevo™ Transforaminal IBF (patient-specific lumbar interbody fusion cages). The Carlsmed aprevo™ device is a 3D-printed, patient-specific fusion cage, which is a spinal implant used to help treat severe spinal deformities in adults who have not improved with standard care. It is custom-made for each patient, using CT scans to better match a patient's anatomy, with the goal of

improving fit and surgical outcomes. The FDA cleared it through the 510(k) pathway based on bench and engineering tests (not trials on humans), comparing it to similar devices already on the market. Despite addressing a serious condition, no studies comparing patient outcomes using this device compared with other devices were submitted to demonstrate it was more effective, even though superior effectiveness is a required criterion of breakthrough devices. This is an example of a breakthrough device that reached the market while lacking any studies of humans (or animals) that proved it met the requirement of being better than the many other spinal fusion cages that are cleared by the FDA – or even if it was as safe or as effective as other spinal fusion cages. Spinal fusion is a risky treatment and can cause serious harm to patients. The cost is not publicly available, but we expect a custom-made implant to cost more, and CMS pays approximately $20,000 more when a spinal implant is "new technology."

Omnipod DASH Insulin Management System. The Omnipod insulin delivery system is a device worn on the arm to calculate and control the amount of insulin provided to people with diabetes. It has been recalled three times due to problems with accuracy, delivering too much or too little insulin. In a 2024 Class I recall (the highest risk recall), the Android app misread certain decimal entries (e.g.,"0.2 units was misinterpreted as “2.0”), which would deliver ten times more insulin than intended and could result in life-threatening hypoglycemia. In the previous (2022) Class I recall, the Omnipod DASH handheld controller faced a battery overheating and fire hazard. A separate Class II (moderate risk) recall involved an error message in the Omnipod 5 Android app that temporarily blocked phone-based insulin dosing, causing therapy delays. These design-related software and hardware flaws affected insulin delivery in dangerous ways. We are glad that these recalls resulted in fixing the problems, but meanwhile, how many patients were harmed? When serious design problems like this are not identified before a device goes on the market, it can be fatal, which is why this device should have been tested on humans before allowing it to be sold.

Summary of Evidence for Breakthrough Devices

The publicly available information on breakthrough devices that are on the market indicates that many were not studied in clinical trials that compared patients with the breakthrough device to patients

with any type of control group, some studies were very small, and many devices were not studied on patients who are likely to be eligible for Medicare (ages 65+) . Although the 510(k) devices were most lacking in clear clinical evidence of safety and effectiveness, especially for patients 65 and older, many of the De Novo and PMA devices also lacked good data on patients who were 65 and older.

Conclusions

Both these bills are intended to help Medicare patients by increasing their access to medical devices. However, in both cases there is a lack of evidence that determines which of these devices have benefits that outweigh the risks for Medicare patients.

Endnotes

1. Zuckerman D.M., Brown P. & Das A. (2014) Lack of Publicly Available Scientific Evidence on the Safety and Effectiveness of Implanted Medical Devices, JAMA Internal Medicine, 174(11): 1781-1787.
2. Kahwati, L. C., Avenarius, M., Brouwer, L., Crossnohere, N. L., Doubeni, C. A., Miller, C., Siddiqui, M., Voisin, C., Wines, R. C., & Jonas, D. E. (2025). Multicancer Detection Tests for Screening. *Annals of Internal Medicine*. https://doi.org/10.7326/ANNALS-25-01877