RE: Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary"

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ECRI Cautions Against CMS Blanket Coverage for FDA-designated Breakthrough Devices

Cites patient safety and efficacy risks to Medicare beneficiaries

ECRI, an independent, nonprofit patient safety and health services research organization, offers the following commentary on the Medicare Coverage of Innovative Technology (MCIT) proposal that would provide national Medicare coverage on the same day as Food and Drug Administration (FDA) market authorization for all breakthrough devices. Such coverage would last for four years. CMS asserts that this new coverage pathway would offer beneficiaries nationwide predictable access to new, breakthrough devices to help improve their health outcomes.

ECRI has assessed the safety and efficacy of medical devices for more than 50 years. ECRI believes that providing Medicare coverage for all devices designated as "Breakthrough" the same day as FDA authorization will

1. pose a patient safety and efficacy risk to Medicare beneficiaries in some cases
2. lower the safety and efficacy evidence requirements used for making coverage decisions
3. disincent the conduct of additional trials focusing on important patient-oriented outcomes in the Medicare population, especially prospective controlled trials

Executive Order 13890, issued by the White House on October 3, 2019, called for streamlining processes for bringing innovative products to market. It specifically called for regulations and guidance to minimize the time and steps between decisions by FDA for approval and the Centers for Medicare and Medicaid Services (CMS) for coverage. It called for evaluation of the parallel FDA and CMS review process with intent to overcome current challenges in use of that process.

The MCIT proposal for same-day decisions on "Breakthrough" devices asserts that this new coverage pathway would offer beneficiaries nationwide predictable access to new, breakthrough devices to help improve their health outcomes. ECRI cautions that such a blanket coverage approach is not in the best interest of patient care and safety, and instead, advocates for the two options that are already in place for early access to new devices: the Parallel Review Process and Coverage with Evidence Development. These tools were developed and implemented to afford Medicare beneficiaries early access to novel technology while also keeping efficacy and patient safety foremost in mind. These vehicles help to ensure that evidence will be gathered on key patient-oriented outcomes so that when new devices diffuse into clinical care, clinicians and patients have a good idea of the potential risks and benefits.

Sixteen devices (*see our assessment of examples in the Appendix*) have received the breakthrough designation. However, not all breakthrough devices are intended for use primarily in the Medicare population. Thus, clinical trials on breakthrough devices not intended primarily for use in the Medicare population typically include few or no patients covered by Medicare, such as the over-65 and disabled populations.

Even when trials on breakthrough devices do include some data on patients aged 65 and older or disabled populations, the available data at the time of FDA approval or clearance is often sparse, relatively short-

term, and significant evidence gaps remain. FDA's regulatory evidence requirements to support device approval or clearance often differ from and are much narrower in scope than the evidence sought by health technology assessment organizations and payors to determine how well a device/procedure works in a given population and how it compares to the standard of care.

Once approved by FDA and covered by Medicare, manufacturers have little incentive to fund controlled studies that will address key evidence gaps on patient-oriented outcomes. FDA has tried to balance early access and the need for more evidence by requiring postmarket studies; however those studies typically do not address key questions that can be answered only by controlled trials. Furthermore, patients are likely to assume that if a device is FDA-approved AND covered by Medicare, sufficient evidence of safety and effectiveness exists for its use in them. In contrast, trials conducted under Coverage with Evidence Development are required to obtain informed consent and to study "whether the item or service meaningfully improves health outcomes of affected beneficiaries who are represented by the enrolled subjects."

In conclusion, ECRI urges CMS not to implement the proposed rule as it stands. If coverage at the time of FDA approval is desired for a breakthrough device, CMS should ensure that sufficient evidence on benefits and risks is available for the Medicare population or use the two vehicles already available to enable early access to Medicare beneficiaries.

Appendix

The following examples on breakthrough devices illustrate our concerns about why Medicare should not automatically cover all breakthrough medical devices on the day of FDA approval:

1. **MiniMed® 670G hybrid closed-loop (HCL) system for treating diabetes type 1**

In September 2016, FDA approved this system and one year after approval, ECRI performed an assessment of available studies that enrolled at least 10 patients. The evidence base consisted of 4 studies that reported on 189 patients—the great majority of whom were adolescents and adults much younger than age 65. These studies were of very short duration—hours to several days long. None compared MiniMed 670G with competing integrated insulin delivery systems. Our conclusion in this assessment was that the evidence overall was inconclusive on the benefits this provided relative to predecessor systems, and for certain age groups, little to no evidence was available.

2. **ExAblate® Neuro (Model 400) magnetic resonance image-guided focused ultrasound (MRgFUS) system for treating medically refractory essential tremor or tremor dominant Parkinson's disease**.

In June 2016, FDA approved the system for essential tremor (ET) and in December 2018 approved a supplement to expand the indication to treat tremor dominant Parkinson's disease in an outpatient setting. ECRI performed assessments of the evidence for these indications.

Six studies reported on 474 patients with essential tremor, many of whom were over 65 years of age. Follow up was relatively short (1 year) and few data were available on comparisons to other therapies, but results on patient-oriented outcomes were consistent across studies, showing a benefit, although five of six studies had a high risk of bias. The overall evidence strength for the conclusion of benefit was low.

For tremor-dominant PD, however, evidence was inconclusive of benefit. Four studies reported on 97 patients and all had high risk of bias due to unbalanced treatment and control groups in a single randomized controlled trial, and absence of controls or blinding in three small case series from single-centers. In addition, studies combined results for patients with different subtypes of PD and patients who underwent different surgeries. Lastly no studies reported on the amount of time patients spent on-medication compared with time off-medication, which is needed to give a clearer picture of Exablate's effectiveness over time.

If the proposed CMS rule had been implemented at the time of the initial FDA approval, then both indications would have been automatically covered by Medicare, given the proposed 4-year automatic coverage window. However, the evidence informing the conclusions of our assessment for each indication differed. In this example, an evidence-based argument could be made that it would be reasonable to consider covering one indication, but not the other. The proposed rule seems to suggest blanket coverage for a "breakthrough device" without considering device-specific indications.

3. **reSET-O® Prescription Digital Therapeutic software app for treating opioid use disorder (OUD)**

In March 2019, FDA granted 510(k) marketing clearance to reSET-O® Prescription Digital Therapeutic, for use with contingency management as part of OUD treatment that includes medication-assisted therapy (MAT). reSET-O delivers cognitive behavioral therapy (CBT) as self-directed education modules that patients are supposed to complete. The app also includes a web-based clinician interface intended to facilitate contingency management, progress monitoring, and feedback. reSET-O is based on a prior internet-based CBT platform used in a treatment center setting. We assessed the available evidence in July 2020.

We identified no studies of reSET-O. Therefore, we expanded our review to include studies of the predecessor platform, although studies had used it in a different care setting, i.e., a treatment center rather than for self-directed therapy with MAT at home. No studies included patients age 65 and older. If automatic coverage were granted under the proposed rule, this software would be available to the Medicare population with no data on this population's ability to use it or on its efficacy. The recent surge in telehealth use during the COVID-19 pandemic has revealed many barriers to effective telehealth use in the Medicare population. Among these barriers are lack of access to computers, lack of knowledge on how to use software and web-based apps on computers or mobile devices, and vision and hearing deficits prevalent in the Medicare population. Thus, automatically providing coverage of software-based telehealth interventions that FDA grants a "breakthrough" designation ignores the need for safety, efficacy, and human factors use data in the Medicare population.