

Partnered Evidence-Based Policy Resource Center

POLICY BRIEF

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Medicare: Coverage with Evidence Development

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Medicare was established in 1965 without a process to determine if and when novel treatments would be covered. Coverage determinations for treatments for which there is not yet adequate evidence of efficacy in the Medicare population remains a challenge today.

To partially address this challenge, in 2006, the Centers for Medicare and Medicaid Services (CMS) issued guidance on Coverage with Evidence Development (CED).¹ This guidance provides protocols for new treatments that require further research before CMS can extend coverage to all Medicare beneficiaries. This brief will explain the CED process and how it might be adapted to inform some coverage decisions within the Department of Veterans Affairs (VA).

Medicare Coverage

CMS has the authority to determine what goods and services Medicare will cover. According to the law establishing the program, Medicare will only cover treatments that are “reasonable and necessary for the diagnosis or treatment of an illness or injury.”²

Medicare determines coverage in two ways: national coverage decisions (NCD) and local coverage decisions (LCD). NCDs apply to all Medicare claims-payment contractors nationwide and are based on rigorous research, expert insight, and public comment. LCDs are developed for services for which there are no existing NCDs and apply only to Medicare contractors in predetermined geographical areas, of which there are sixteen.^{2,3}

If CMS determines a service will be covered by Medicare, eligible beneficiaries are able to receive the treatment or procedure from a participating health care provider. Depending on the service rendered, the beneficiary may be responsible for some or none of the associated cost.

CED Protocol

The CED process (Table 1) is used for Medicare national coverage decisions that CMS determines require additional clinical data collection to inform a final ruling. CED is restricted to ensure no overlap with other federal agencies’ jurisdiction. For example, CED trials cannot duplicate the U.S. Food and Drug Administration’s authority (e.g. testing toxicity) or the National Institutes of Health’s research (e.g. developing new treatments).¹

To be approved, the treatment and CED protocol must already be well supported by the literature—deemed safe and effective in a study population—and methodologically sound. A CED will determine whether the treatment will “meaningfully improve” the health of Medicare beneficiaries.¹

When beneficiaries receive treatment under CED protocol, they will participate in a CMS-approved clinical trial. CMS must publicly publish the study findings—both positive and negative—within a year of study end dates. The CED is deemed complete once CMS reviews the study findings and allows beneficiaries to receive coverage without participating in CMS-approved research study. Alternatively, CMS can decide to redevelop or reconsider the CED if the collected evidence is insufficient to expand coverage eligibility.¹

The entire process is meant to be fully transparent. Members of the public are welcome to review proposed CEDs, comment on study design, meet with CMS staff, or request a decision reconsideration based on study results.¹

Table 1: CED Protocol

- **For NCDs requiring additional data collection**
- **Treatment must be well-supported by current literature**
- **Determines if treatment “meaningfully improves” health of beneficiaries**
- **Clinical trial results are published at the end of CED period**
- **Process is publicly transparent**

The CED protocol does not consider the value of treatments, just their effectiveness. This is consistent with Medicare’s troubled history with value-based decision making.⁴ Past attempts to incorporate value-based analysis into coverage decisions have been unsuccessful. For example, after a decade of internal review, regulations proposed in 1989 that would have added cost-effectiveness as a coverage criterion were withdrawn.⁵

Overall, CED is not a common way CMS determines Medicare coverage; according to the CMS website, there have been two dozen therapies considered via the CED protocol (Table 2).⁶ Of these, only one has emerged from CED and now is covered by Medicare—CPAP for obstructive sleep apnea.

Table 2: Treatments/Services Considered by CED Protocol⁶

Treatment/Service	CED Initiation Date
FDG PET and Other Neuroimaging Devices for Dementia	September 2004
Cochlear Implants	April 2005
Off-label use of Colorectal Cancer Drugs	April 2005
Home Oxygen for COPD	March 2006
CPAP for Obstructive Sleep Apnea*	March 2007
Artificial Hearts	May 2008
Pharmacogenomic Testing for Warfarin Response	August 2008
NaF-18 PET for Bone Metastasis	February 2010
Allogenic Hematopoietic Stem Cell Transplant for MDS	August 2010
Home Oxygen for Cluster Headache^	January 2011
Magnetic Resonance Angiography and Magnetic Resonance Imaging	February 2011
Extracorporeal Photopheresis for Bronchiolitis Obliterans Syndrome Following Lung Transplant	April 2012
Transcatheter Aortic Valve Replacement	May 2012
TENS for Chronic Low Back Pain^	June 2012
Autologous Platelet-rich Plasma	August 2012
Amyloid PET	September 2013
Percutaneous Image-guided Lumbar Decompression for Lumbar Spinal Stenosis	January 2014
Transcatheter Mitral Valve Repair	August 2014
Allogenic Hematopoietic Stem Cell Transplant for Multiple Myeloma	January 2016
Allogenic Hematopoietic Stem Cell Transplant for Myelofibrosis	January 2016
Allogenic Hematopoietic Stem Cell Transplant for Sickle Cell Anemia	January 2016
Percutaneous Left Atrial Appendage Closure (LAAC)	February 2016
Leadless Pacemakers	January 2017
<i>*Covered as of March 2008</i>	
<i>^No approved clinical trials</i>	

Recent CED Decisions

In 2019, CMS decided to expand access to chimeric antigen receptor (CAR) T-cell therapy and transcatheter aortic valve replacement (TAVR) through CED. To illuminate the initiation of CED and subsequent coverage decisions and revisions, we provide additional details about these two cases in the following subsections.

Chimeric Antigen Receptor T-cell Therapy

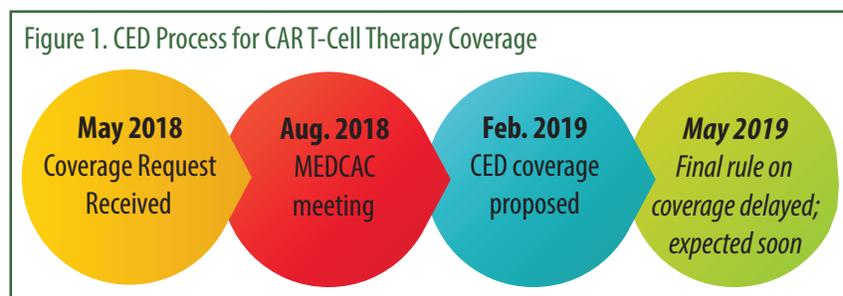
CAR T-cell therapy provides an example of how CMS initiates the CED process (Figure 1).

CAR T-cell therapy is an emerging form of cancer therapy that uses the patient's own immune system to target cancer. A sample of the patient's T-cells are collected and genetically engineered in the laboratory to better detect and attack cancer cells.⁷ Use of CAR T-cell therapy has been limited to clinical trials and only a few specific cancer types, for which it has been quite successful.⁸ However, a notable concern is cost. A single dose of CAR T-cell therapy can cost almost \$500,000, excluding associated expenses such as hospital admissions or other medications.⁹

CMS has approached coverage of CAR T-cell therapy for Medicare beneficiaries cautiously. It received a formal request in May 2018 to consider CAR T-cell therapy and held a Medicare Evidence Development & Coverage Advisory Committee (MEDCAC) meeting on the treatment in August 2018.¹⁰

In February 2019, CMS proposed CED coverage for the cancer therapy. Specifically, CMS will pay for treatment when it is offered in a CMS-approved registry or clinical trial. Medicare patients must be clinically monitored for two years afterwards to document the efficacy and long-term effects of treatment. CMS will then use the trial's findings to inform coverage determinations outside the confines of a clinical trial.¹⁰

The February 2019 announcement allowed for 30 days of public comment and CMS will issue its final rule on the CED trial in May 2019.¹⁰ CMS was set to issue its final rule on the CED trial in May 2019, but, on May 17, 2019, it delayed announcing a decision for undisclosed reasons. CMS will release a final rule in the coming months.^{10,11}



Transcatheter Aortic Valve Replacement

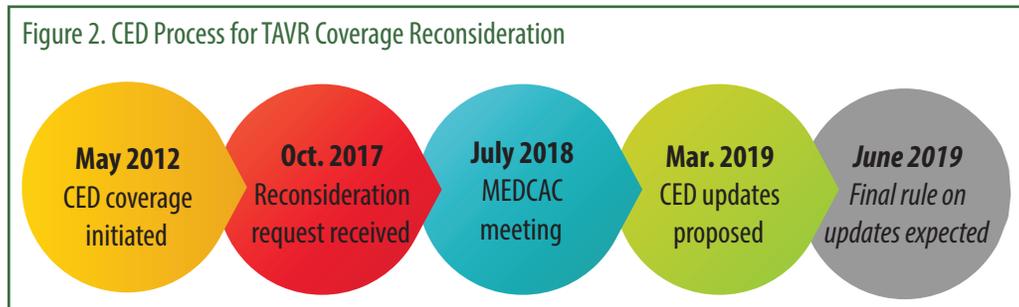
TAVR provides an example of how CMS reconsiders its CED decisions based on current CED evidence collection and stakeholder input (Figure 2).

TAVR is a minimally invasive procedure to replace the aortic valve of the heart when the patient is diagnosed with aortic stenosis. It is used as a safer alternative to open heart surgery. CMS initiated CED coverage for TAVR in May 2012 and over 500 hospitals nationwide have established TAVR programs since.¹²

Today, national Medicare coverage of TAVR is still governed under CED protocol. However, in October 2017, CMS received a formal request from a group of physicians to reconsider the CED coverage decision. MEDCAC held meeting in July 2018 on the request.^{13,14}

With input from MEDCAC, CMS proposed updates to the TAVR CED guidelines in March 2019, focused particularly on the guidelines for hospitals that wish to establish TAVR programs. The proposed updates will require hospitals and providers to meet procedure volume requirements. However, there will be additional consideration for patient safety and care quality with the possibility of later replacing the procedure volume requirement altogether in favor of other metrics.¹²

The March 2019 announcement allowed for 30 days of public comment and a final rule will be issued in June 2019.¹² Beneficiaries will continue to receive TAVR in CMS-approved clinical trials, accounting for any CED protocol revisions.



VA Policy Implications

Novel treatments are often more complex and/or more expensive than those that are currently available. Just as CMS must determine whether and how it will cover new treatments for Medicare beneficiaries, VA must do the same for Veterans.

An advantage of VA is the robust collaboration between operations and research; CED could be a natural continuation of that relationship. CED would allow VA to trial new treatments for a predetermined time frame while simultaneously collecting valuable Veteran-specific evidence on treatment efficacy and impact.

Additionally, VA could incorporate value into its CED protocol, reflecting the fact that it operates on a fixed budget, unlike Medicare. Doing so could ensure that new treatments receive coverage if they are both clinically effective and offer superior value compared to current treatments. A value-based CED approach could also provide VA more leverage during price negotiation with treatment or service suppliers.

A recent example is a VA-sponsored randomized clinical trial evaluating the use of service dogs for PTSD patients. In this trial, Veterans with PTSD are randomly assigned either a service dog or an emotional support animal. Results from the study—evaluating various measures of utilization, costs, and well-being—will then be provided to the Institute for Clinical and Economic Review (ICER). ICER will develop a model to assess the value of providing service dogs to Veterans relative to emotional support animals and to the existing standard of care for PTSD.

If VA adopts a value-based CED approach to new treatments, it will also need to determine how to proceed should treatments fail to meet VA standards of clinical effectiveness and/or value. VA must develop guidelines for discontinuing ineffective or low-value treatments in a way that does not negatively impact Veterans' care.

Medicare's CED process is an evidence-based approach to novel clinical treatments through which CMS aims to ensure new treatments are effective and safe for Medicare beneficiaries. With protocols in place for discontinuing unsuccessful treatments, VA stands to benefit from a similar approach as it seeks to offer Veterans the highest quality, highest value healthcare.

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About PEPReC Policy Briefs

This evidence-based policy brief is written by the Partnered Evidence-based Policy Resource Center (PEPReC) staff to inform policymakers and VHA managers about the evidence regarding important developments in the broader health system and economy. PEPReC, the Partnered Evidence-based Policy Resource Center, is a QUERI-funded resource center that collaborates with operational partners to design and execute randomized evaluations of VHA initiatives, develops and refines performance metrics, and writes evidence-based policy briefs.