NATIONAL HEALTH COUNCIL

August 21, 2023

Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 212441

RE: Coverage with Evidence Development Proposed Guidance Document

Dear Administrator Brooks-LaSure:

The National Health Council (NHC) appreciates the opportunity to provide input to the Centers for Medicare and Medicaid Services' (CMS') proposed guidance on coverage with evidence development (CED).

Created by and for patient organizations more than 100 years ago, the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, sustainable, equitable health care. Made up of more than 150 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic drug, and payer organizations.

Making sure that the coverage decision process works in favor of patients efficiently and safely having access to innovative treatments is a priority for the NHC, and we appreciate CMS's attention to improving the process. We urge CMS to take a measured and balanced approach towards implementing CED in a way that enhances access to the greatest extent possible.

CED, which could be a useful tool in getting innovative products to patients more quickly, can also unnecessarily restrict access to treatments that would otherwise be covered by Medicare. The burden associated with access driven by CED studies can make it prohibitive or otherwise not pursued by treating physicians. It can also result in inequitable access to treatments. For instance, if CED studies are limited to academic institutions that are not geographically located in diverse areas, it may influence the demographics of the participants. The rigors of CED studies might also limit access for people from marginalized communities that may have economic or transportation barriers to participation. A 2021 study found that one of the top five barriers to increasing participation of patients from historically underrepresented racial and ethnic backgrounds in clinical trials was "time and resource constraints associated with

participation."[1] We recommend that the CED pathway include an exception process in place for those patients in underserved communities that may not be able to access an approved study site or have other issues with access.

There is still a lack of predictability and understanding of when CED will be used, and the more clarity that CMS provides, the more patients and advocates will be able to partner with CMS to provide input to assure CED is used appropriately.

The Relationship Between FDA Approval and CED

Further clarity on the use of CED is particularly important because of the variety of paths that treatments and devices may take to enter the approval process. The relationship between FDA approval and the use of CED can be complicated. For drugs, there is a straightforward pathway to coverage after FDA approval. Once safety and efficacy have been approved by the FDA — and CMS has reviewed the reasonableness and necessity of the treatment — coverage should very rarely need further investigation. For some devices, the pathway is different, and CED may be used to gather needed data to meet more robust standards.

On one hand, the use of CED for FDA-approved medicines has raised concerns about delays in access to treatment and geographic variation in access to treatment. For example, CMS has implemented CED for novel amyloid beta-targeting therapies for Alzheimer's disease even when these therapies are being used according to their FDA-approved label and accepted medical practice, and continued CED even once products have transitioned from accelerated approval to traditional approval. Use of CED in this manner threatens the FDA's authority on the question of efficacy. Patients may end up not benefiting from the accelerated approval pathway, as originally intended, which was designed to speed access to new medicines. Therapies approved using the accelerated approval pathway are not considered experimental. They have been evaluated rigorously using a surrogate endpoint so that individuals facing serious or life-threatening conditions with great unmet needs can access them at the earliest possible moment. When CED is implemented for novel FDA-approved medicines, patient access suffers, as patients may only access these medicines through sites that are participating in the CMS-approved CED studies and themselves have no option but to consent to participation.

On the other hand, there are instances where the use of CED can help further needed evidence collection, particularly in cases where standards for FDA approval are less stringent. Medical devices that received FDA clearance through the 510k pathway are a good example of where these trials may be useful to establish additional data for efficacy in order to receive coverage. The 510k pathway allows medical device manufacturers to demonstrate that their technology works the same way as an existing

[1] Clark LT, Watkins L, Piña IL, et al. Increasing Diversity in Clinical Trials: Overcoming Critical Barriers. *Curr Probl Cardiol*. 2019;44(5):148-172. doi:10.1016/j.cpcardiol.2018.11.002

technology to gain approval. If CMS would like more data to establish coverage for these devices, the CED process allows collaboration, so that the product manufacturer can gather the right data to meet CMS's needs. In these instances, impacts to beneficiary access are less acute because by definition, an analogous device is already on the market. This is not true in the case of drugs that have a more robust FDA approval process to demonstrate safety and efficacy.

Patient Engagement

CMS currently seeks input on proposed coverage policies through the National Coverage Determination (NCD) process, but patients do not have a way to directly engage with the agency on the choice to apply CED requirements. As with all health care decisions that CMS and others in the health care ecosystem influence, the NHC recommends that CMS consult patients on how imposition of CED might impact:

- Beneficiary access to treatment (e.g., geographic barriers);
- Beneficiary health and outcomes (e.g., from delayed access to care); and
- Beneficiary and caregiver experience (e.g., quality of life and other factors).

If CMS determines that it will utilize CED, it should also consult patients on study design protocols and outcomes of relevance to transitioning to full coverage for the product or service.

The need for patient engagement in trial design, choice of outcomes most important to patients, and other parts of the CED decision making process is not directly addressed in this guidance. In the past, the primary role of patients has been limited to their role as study subjects. Understanding their ability and willingness to participate in studies is critical. We need to make sure that study designs are as least burdensome as possible and support the patient's successful participation in trials. If there are issues with participation, the patient perspective can also identify barriers that can be overcome. In both examples, engaging patients both in study design and implementation will result in better outcomes. In addition, when designing studies, identifying measures and outcomes that matter to patients is another key area of engagement. Over the past two decades, stakeholders have collaborated to develop best practices for identifying concepts important to patients and developing corresponding patient-centered outcome measures. The NHC urges CMS to make sure that all aspects of the CED process properly engage patients.

Reevaluation of CED

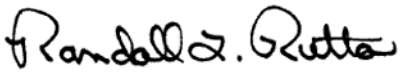
One other area of needed clarity is the protocol for reevaluation as treatments are in the CED process and how the transition to NCD may occur. In defining CED study protocols, CMS should be clear about the specific timing for evaluation of study data and reconsideration of CED and the criteria under which CMS will remove the CED requirement for coverage. In addition, when CMS determines that the data support removal of a CED requirement, CMS should issue a revised NCD that specifies full

coverage for the product or service, as this is the best way to ensure consistent access for Medicare beneficiaries with no disruption in care.

Conclusion

Please do not hesitate to contact Eric Gascho, Senior Vice President of Policy and Government Affairs, if you or your staff would like to discuss these issues in greater detail. He is reachable via e-mail at egascho@nhcouncil.org.

Sincerely,



Randall L. Rutta
Chief Executive Officer