



November 2, 2020

The Honorable Seema Verma  
Administrator  
Centers for Medicare & Medicaid Services  
Department of Health and Human Services  
Attention: CMS-1734-P  
P.O. Box 8016  
Baltimore, MD 21244-8016

Re: CMS-3372-P  
**Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and  
Definition of “Reasonable and Necessary”**

Dear Administrator Verma:

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for all Americans living with or caring for someone with a rare or ultra-rare condition. We strive to amplify the patient and caregiver voice in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic.

The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative that brings together rare cancer patient organizations. RCPC gives participants a platform for focusing specifically on systemic reimbursement barriers and emerging landscape changes that impact new product development and treatment access for rare cancer patients. It is the only rare cancer coalition developed to focus on and respond to reimbursement, access and value issues across the rare cancer community. Working within the Haystack Project enables RCPC participants to leverage synergies and common goals with rare and ultra-rare patient advocates and optimize advocacy in disease states where unmet need is high and treatment inadequacies can be catastrophic.

Haystack Project and RCPC (collectively, “Haystack”) appreciate the opportunity to comment on the above-referenced Proposed Rule. For our patient communities, the emergence of new, innovative therapies targeting specific disease mechanisms offer renewed hope for treatment options, and even a cure to the life-limiting and life-



threatening conditions they face. This hope, however, is tempered by our concern that broad health policy refinements will drive unintended consequences for our patients and impede patient access to both novel, treatments addressing very rare disorders and rare cancers and off-label use of existing treatments to reduce disease burden when an FDA-approved treatment is not available. Our comments:

- Provide a brief background on the unique concerns of patients with rare and ultra-rare diseases and rare cancers;
- Urge CMS to pursue its proposed Medicare Coverage of Innovative Technology (MCIT) initiative through a National Coverage Determination rather than as a codified regulation;
- Urge CMS to abandon its proposal to codify a definition of “reasonable and necessary;”
- Recommend that, to the extent CMS intends to finalize a codified definition of “reasonable and necessary,” it should provide greater granularity on the intended scope of applicable items and services and re-issue a proposed rule that clearly notifies and seeks comment from all relevant stakeholders;
- Urge CMS to include use of commercial payer coverage policies as an optional category of evidence that can be considered in expanding coverage, rather than as a potential substitute for MAC or CMS evidence-based decisions.

## **Background**

Over 35 years ago, Congress recognized that commercial realities associated with research and development discouraged innovation in treating serious medical conditions affecting small populations. Countless lives have been improved, or saved, by new therapies stimulated by the set of statutory incentives for orphan drugs. Although millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure, there are many for whom treatments that are already available or in the pipeline are out of reach due to the realities of current reimbursement structures.

Innovation in how we understand and address disease mechanisms are currently advancing at a previously unthinkable pace. Targeted cancer treatments, gene therapy and regenerative medicine, and immunologic approaches to rare, serious, and life-threatening conditions give renewed hope to patients and their caregivers. Novel treatments have, however, been accompanied by increased concerns that the treatments we need will be accompanied by access restrictions that limit their real-world utility in addressing disease burden for all patients that are candidates for treatment. For patients without an FDA-approved treatment option, reliance on off-label use of existing products is essential to



manage symptoms, reduce disease burden, and, for some patients, can slow symptom progression. These patients face a disproportionate set of hurdles in accessing treatment due to coverage policies among public and private payers that tend not to account for off-label use in rare and ultra-rare disease populations.

**Haystack urges CMS to pursue its proposed Medicare Coverage of Innovative Technology (MCIT) initiative through a National Coverage Determination (NCD) rather than as a codified regulation.**

Haystack supports CMS' proposed creation of a mechanism through which Medicare beneficiaries would have rapid, reliable access to breakthrough medical devices. The MCIT proposal is an appropriate exercise of CMS' authority to determine the circumstances under which a newly-marketed medical device would be presumed reasonable and necessary and qualify for coverage. Haystack believes, however, that a "blanket" NCD would be a more appropriate avenue for the MCIT pathway because CMS' proposal is a determination of medical necessity with clearly outlined eligibility criteria.

- The MCIT pathway provides up to 4 years of national coverage to newly FDA market authorized breakthrough devices;
- Breakthrough devices that received FDA market authorization no more than 2 calendar years prior to the effective date and thereafter will be eligible for coverage for claims submitted on or after the effective date;
- The 4-year period starts on the date of FDA market authorization;
- Covered items and services could include the device, reasonable and necessary surgery to implant the device, if implantable, related care and services (for example, replacing reasonable and necessary parts of the device such as a battery), and coverage of any reasonable and necessary treatments due to complications arising from use of the device; and
- Coverage would begin on the same date the device receives FDA market authorization.

We support the proposal to start the 4-year coverage period on the date of FDA market authorization as reasonable for new devices, and urge CMS to apply the same 4-year coverage period to all MCIT-eligible devices, including those that were marketed in the 2-year period prior to implementation of CMS' decision. The MCIT pathway was designed specifically to respond to coverage uncertainties and hurdles experienced in connection with breakthrough devices to date. We expect that those hurdles may have substantially impeded meaningful access to breakthrough devices marketed within the 2-year period for MCIT eligibility, and believe the full 4-year coverage period would provide patients and CMS with an opportunity to collect data on real-world use within the Medicare population.



Haystack is also concerned that two of CMS' MCIT proposals are likely to trigger unintended consequences that undercut the over-arching goals of the MCIT pathway, particularly for individuals with rare and ultra-rare conditions.

- First, CMS proposes that if a breakthrough device is the subject of an NCD, the device would not be eligible for MCIT. We are concerned that this provision injects uncertainty into the MCIT pathway by opening the possibility that CMS could initiate an NCD for a soon-to-be-marketed device and foreclose coverage under an MCIT pathway. CMS could, however, achieve the same goals of enabling access to breakthrough devices with a single “breakthrough device MCIT” NCD. The rationale CMS articulated to support a **regulation** establishing national coverage of breakthrough devices as “reasonable and necessary” also supports **national coverage within an NCD**. The NCD mechanism would also provide CMS with greater flexibility in identifying devices subject to existing NCDs rather than the breakthrough device MCIT NCD.
- Second, CMS proposes requiring that devices must be used according to FDA approved or cleared indication for use, ***with off-label uses rendered non-covered on a national basis***. Haystack is concerned that individuals with rare and ultra-rare diseases often manage disease symptoms through off-label use of products approved for more common conditions as part of the practice of medicine within subspecialties caring for these patients. Medicare beneficiaries often encounter a labyrinth of documentation, prior authorization, and/or appeals processes to gain access to the care they need. **A regulatory barrier to coverage for off-label uses of breakthrough devices would likely make access to these products far more complex than it is for non-breakthrough devices**. Given that the Medicare appeals process would not permit inquiry beyond verifying that the use was off-label and within the regulatory coverage prohibition, the only mechanism for access would be a challenge to the regulation itself. The NCD mechanism to the MCIT pathway would instead enable CMS to place off-label uses within contractor discretion, and if appropriate, permit device- and patient-specific challenges to NCD-related noncoverage of off-label uses. It would also avoid uncertainties associated with an MCIT pathway that is at odds with the health care needs of rare and ultra-rare patients.

We therefore strongly suggest the NCD mechanism as a more appropriate avenue for the MCIT pathway that would mitigate the potential for unintended constraints on access associated with a codified regulation.



**Haystack urges CMS to abandon its proposal to codify a definition of “reasonable and necessary.”**

As CMS articulated in its Proposed Rule, determinations of whether or not items and services are “reasonable and necessary” can be made (1) at the beneficiary level through the claims processing mechanism and any pre- or post-payment review; (2) at the “local” level by a Medicare Administrative Contractor (MAC) initiating a Local Coverage Determination (LCD); or (3) on a national level through the NCD process. Haystack is

concerned that CMS’ proposal to codify a single set of criteria within a definition for “reasonable and necessary” conflates the appropriate criteria and intended purpose for an NCD with those of Local Coverage Determinations and patient-specific claim adjudications.

**Haystack has significant concerns that uniformity is inappropriate given the refinements CMS proposes and the fact that these decisions are not monolithic; they vary in scope from the individual patient claim adjudication to population-based decisions on a “local” or “national” level.**

The Medicare Program Integrity Manual guides MACs, the Comprehensive Error Rate Testing (CERT) Contractor, Recovery Auditors, and Uniform Program Integrity Contractors (UPICs) in appropriate criteria for establishing medical necessity at the claims adjudication and review levels, including that:

- It is safe and effective;
- It is not experimental or investigational; and
- It is appropriate, including the duration and frequency in terms of whether the service or item is:
  - o Furnished in accordance with accepted standards of medical practice for the diagnosis or treatment of the beneficiary's condition or to improve the function of a malformed body member;
  - o Furnished in a setting appropriate to the beneficiary's medical needs and condition;
  - o Ordered and furnished by qualified personnel; and,
  - o One that meets, but does not exceed, the beneficiary's medical need.<sup>1</sup>

MACs apply criteria to establish the conditions under which an item or service is reasonable and necessary at the contractor-wide level through the LCD process. This has

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<sup>1</sup> Medicare Program Integrity Manual, Chapter 3, Section 3.6.2.2 - Reasonable and Necessary Criteria. <https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/Downloads/pim83c03.pdf>



historically been a program integrity mechanism through which MACs address over-utilization with claims processing “edits” that deny payment when claims do not meet the established medical necessity criteria within the LCD. The LCD process can also be initiated through requests from beneficiaries, health care professionals, or other interested parties within the contractor’s jurisdiction. The criteria for LCD analyses of “reasonable and necessary” are identical to those listed above for claim-specific determinations, with an additional criteria that the item or service be “at least as beneficial as an existing and available medically appropriate alternative.” CMS’ proposal did not clearly state that this LCD criterion would be added as part of “reasonable and necessary” determinations made by MACs at the claims processing and pre- and post-payment review levels. It is, therefore,

unlikely that stakeholders will have the opportunity to assess and communicate to CMS what, if any, impact this additional criterion might have on beneficiary access.

Even more concerning is CMS’ proposal to change the perspective from which “appropriateness” is determined **from the beneficiary** receiving treatment to a **population-level** by inserting “for Medicare patients” into the appropriateness prong on the inquiry. In fact, the Social Security Act (SSA) establishes a **beneficiary-specific** entitlement to benefits:

SEC. 1832. [42 U.S.C. 1395k] (a) The benefits provided to an individual by the insurance program established by this part shall consist of—

(1) entitlement to have payment made to him or on his behalf (subject to the provisions of this part) for medical and other health services, except those described in subparagraphs (B) and (D) of paragraph (2) and subparagraphs (E) and (F) of section 1842(b)(6)

Medicare and its MACs have consistently viewed claim-specific medical necessity adjudications within the context of the **beneficiary’s** condition, including any comorbidities or complicating factors. The Medicare Program Integrity Manual instructions related to claim adjudication similarly reflect the view that **each Medicare beneficiary** is entitled to medical care that is reasonable and necessary for the diagnosis or treatment of the **beneficiary’s** condition or to improve the function of a malformed body member.

We are concerned that CMS’ insertion of a “Medicare patients” perspective will drive **population-based** evidentiary analyses and conclusions that, while potentially appropriate in proposed draft guidance documents related to NCDs under Section 1862(l)(1), are **in conflict with longstanding interpretations of each beneficiary’s entitlement to reasonable and necessary care.**



Haystack is similarly concerned that the concept of “Medicare patients” as a distinct and definable subpopulation may neglect consideration of the non-elderly Medicare beneficiaries that make up approximately 15% of the overall Medicare population and include:

- People under age 65 who are disabled and have received SSDI payments for 24 months; and
- People under age 65 who are diagnosed with end-stage renal disease (ESRD) or amyotrophic lateral sclerosis (ALS).<sup>2</sup>

**This shift in “reasonable and necessary” analyses will have a disproportionate adverse impact on patients with rare and ultra-rare conditions, rare cancers, and multiple chronic conditions.** The regulatory provision would apply to and limit the types of evidence beneficiaries could submit to establish that the care they seek is medically necessary to evidence establishing that it is appropriate for “Medicare patients” **as a whole** rather than that it is appropriate care for **the beneficiary’s** condition or unique set of conditions. This population-level evidentiary determination would, pursuant to the newly-codified regulation, be a question of fact for which deference would likely be afforded to the MAC or Departmental Appeals Board. At a minimum, we expect that Medicare beneficiaries with rare and ultra-rare conditions, rare cancers, and idiosyncratic combinations of chronic conditions, comorbidities, and risk factors would have to engage higher levels of appeals more frequently when they already face significant barriers to access. More likely, **we expect that our community of patients will once again be lost in population-based analyses, and appropriate care will simply be denied.** For patients with rare and ultra-rare conditions, codifying the refined, singular set of criteria for “reasonable and necessary” determinations as a population-level inquiry could foreclose all meaningful avenues for challenging claim denials, leaving a direct challenge to the regulation itself as the only option.

Finally, Section 1862(l) of the SSA directs the Agency to utilize a notice and comment process to develop guidance documents, not regulations, on factors considered in “reasonable and necessary” determinations made within the NCD process.

1862 l) NATIONAL AND LOCAL COVERAGE DETERMINATION PROCESS.—

- (1) FACTORS AND EVIDENCE USED IN MAKING NATIONAL COVERAGE DETERMINATIONS.—The Secretary shall make available to the public the factors considered in making national coverage determinations of whether an item or service is reasonable and necessary. **The Secretary shall develop guidance documents to carry**

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<sup>22</sup> <https://www.kff.org/medicare/issue-brief/an-overview-of-medicare/>



**out this paragraph in a manner similar to the development of guidance documents under section 701(h) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 371(h)).** [*emphasis added, citations omitted*]

As Haystack noted above, the Medicare Program Integrity Manual distinguishes claim-specific medical necessity determinations from LCDs with respect to the criteria for consideration. Congress also recognized that factors and criteria could, and perhaps should, differ based upon the scope and breadth of the decision, and retain a measure of inherent flexibility, by directing CMS to develop “guidance documents” specific to the “reasonable and necessary” requirement applied to NCDs.

We strongly urge CMS to abandon its proposal to codify a singular set of criteria for establishing that an item or service is “reasonable and necessary.”

**Haystack urges CMS to provide greater granularity on the intended scope of applicable items and services within the proposed definition of “reasonable and necessary” and re-issue a proposed rule that clearly notifies and seeks comment from all relevant stakeholders.**

Haystack and its patient advocacy organizations have encountered a significant amount of divergence among stakeholder understanding on the scope of CMS’ proposed definition of “reasonable and necessary.” We have heard from stakeholders suggesting that the Proposed Rule applies only to medical devices, and not to drugs, laboratory tests, or surgical procedures. The proposed placement of the regulatory text suggests that this interpretation is correct. We have also encountered patient advocacy organizations and other stakeholders that urge us to view the proposal as broadly applicable based on discussions with CMS.

We are concerned that when, as here, stakeholders are unable to analyze a proposed rule and reach some level of common understanding on its scope and impact, the notice and comment process falls short of its purpose. Given the potential impact CMS’ proposal could have on patients, providers, and manufacturers, it is essential that all stakeholders, particularly patients and patient organizations have the opportunity to offer meaningful input. Haystack, therefore, urges that, to the extent that CMS has determined to proceed with a codified definition of “reasonable and necessary,” it do so through a re-issued proposed rule that takes into account comments received, and provides ample opportunity for all stakeholders to consider its impact on patients, and any safeguards required to ensure that any unforeseen impediments to patient access are addressed and resolved with minimal burden to patients and providers.





**Haystack urges CMS to include use of commercial payer coverage policies as an optional category of evidence that can be considered in expanding coverage, rather than as a potential substitute for MAC or CMS evidence-based decisions.**

CMS proposes that an item or service would be “appropriate for Medicare patients” if it is “covered in the commercial insurance market, except where evidence supports that there are clinically relevant differences between Medicare beneficiaries and commercially insured individuals.” Haystack recognizes that CMS seeks to implement the directives contained in the October 3, 2019 “Executive Order on Protecting and Improving Medicare for Our Nation's Seniors” to make coverage of breakthrough medical devices “widely available, consistent with the principles of patient safety, market-based policies, and value for patients.”

Haystack notes that CMS’ stated intent is to provide an additional avenue for demonstrating that an item or service is appropriate. However, we are extremely concerned that broad use of commercial coverage policies would likely **lead to broad incorporation of coverage restrictions**. Commercial insurers, unlike the Medicare program, often implement coverage policies for each new drug or device for which utilization is likely to reach a sufficient threshold. Many, if not most, coverage policies for higher-cost products are implemented through a maze of potentially complex set of documentation and prior authorization requirements, which to our patients and their physicians, seem designed to deter access. Grafting these policies, including their restrictions, onto the Medicare program would result in a significant number of claims requiring manual processing, denials pending documentation. We expect that there would also be an increase in use of Advance Beneficiary Notice of Noncoverage (ABN) as a substitute for commercial payer use of prior authorization, which is a monumental deterrent to patients.

Haystack’s specific comments on use of commercial plan coverage policies depends on the scope of items and services to which it would apply. We expect that the alternative mechanism for establishing that a **device** is “appropriate” may streamline access within the spirit of the Executive Order. If, however, it is broadly applied across the Medicare program to **all items and services** for which there is no applicable NCD or LCD, access constraints and increased provider burden are very likely.

Haystack is also concerned about unintended impact on the reconsideration and appeals processes that would follow codification of this proposal, particularly if it applies broadly across Medicare. We urge CMS, once again, to clarify the scope and impact of its proposal, and consider adopting use of commercial coverage policies, through subregulatory guidance, as an acceptable evidentiary resource for MACs developing LCDs in response to



stakeholder requests. We believe this option appropriately balances an interest in market-based approaches with beneficiary access to innovative treatment options.

### Conclusion

Haystack Project appreciates the opportunity to offer its comments and suggestions in connection with CMS' proposed MCIT pathway and definition of "reasonable and necessary." We look forward to working with you to ensure that all beneficiaries have access to the care they need, no matter how rare their disease or condition.

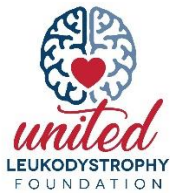
We look forward to a continuing dialogue to ensure that all patients are able to receive appropriate care, no matter how rare their disease or condition. If you have questions or need further information, please do not hesitate to contact Saira Sultan at 202-360-9985.

Sincerely,



# HAYSTACK PROJECT

The Voices of Rare & Ultra Rare



FOXP1 SYNDROME



