ASTCT
American Society for
Transplantation and Cellular Therapy

November 2nd, 2020

The Honorable Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS–3372–P
P.O. Box 8013
Baltimore, MD 21244–1850

SUBMITTED ELECTRONICALLY VIA REGULATIONS.GOV

Re: Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of ‘‘Reasonable and Necessary’’

Dear Administrator Verma:

The American Society for Transplantation and Cellular Therapy (ASTCT) is pleased to offer the following limited comments on the Medicare Coverage of Innovative Technology (MCIT) proposal.

The ASTCT is a professional membership association of more than 2,200 physicians, scientists and other health care professionals promoting blood and marrow transplantation and cellular therapy through research, education, scholarly publication and clinical standards. The clinical teams in our society have been instrumental in developing and implementing clinical care standards and advancing cellular therapy science, including participation in trials that led to recent FDA approvals for Chimeric Antigen Receptor T-cell (CAR-T) therapy.

The ASTCT commends CMS' interest in "ensuring Medicare beneficiaries have access to new cures and technologies that improve health outcomes."

The members of ASTCT have been focused on innovation in the treatment of hematologic malignancies, hematologic disorders and other immune system diseases for more than 25 years. As hematopoietic cell transplantation (HCT) has been expanded into the Medicare beneficiary population and into new diseases, our providers have experienced the same issues identified by CMS as potential barriers to breakthrough device adoption. CAR-T, despite being an FDA-approved biologic, was subject to the National Coverage Analysis (NCA) process, introducing an extensive (15 month) period of coverage uncertainty before a final determination was made. HCT experiences an on-going national coverage variation in coverage due to Medicare Administrative Contractor (MAC) discretion and claim-by-claim adjudication, as there is not an umbrella NCD or set of LCDs for each of the standard transplant indications that are routinely

covered by commercial insurance.[1] The National Coverage Analysis process is lengthy, burdensome to the provider community, and disproportionately precludes Medicare beneficiaries from accessing treatment options available to the commercially insured population, stratifying access to innovation by age. Additionally, the NCA process is being misappropriated by Medicare Advantage contractors as a mechanism to decrease their financial liabilities in relation to innovative technology. When Medicare finalizes a positive NCD and verifies that a new therapy meets the significant cost criterion, MA plans are relieved of the financial responsibility for those therapies for two years, or until the MA bid and payment rates can be adjusted.[2] This linkage creates an incentive for Medicare Advantage Plans to request NCA for financial reasons, instead of substantial clinical concerns.[3]

CMS notes that it is bringing the MCIT proposal forward as there is no other mechanism that will provide "immediate, predictable coverage." We agree that Medicare beneficiaries should be afforded early and predictable coverage of FDA-approved breakthrough therapies, ***regardless of whether they are devices or drugs/biologics***, and encourage CMS' efforts to improve its current processes. **However, we have strong concerns that what is being proposed in this rule will not meet these objectives**. Specifically, offering interim coverage to a specific category of therapies could preferentiate use of those therapies over other established modalities of care that may not have clear NCDs or be able to seek FDA approval through the breakthrough therapy pathway(s).

While CMS points specifically to the mention of devices in Executive Order 13890 (E.O. 13890) ''Executive Order on Protecting and Improving Medicare for Our Nation's Seniors,'' we note that E.O.13890 indicated a broader scope of consideration:

> *[T]he Secretary shall propose regulatory and sub-regulatory changes to the Medicare program to encourage innovation for patients by streamlining the approval, coverage and coding processes so that* ***innovative products*** *are brought to market faster, and so that such products, including breakthrough medical devices and advances in telehealth services* ***and similar technologies*** *[emphasis added], are appropriately reimbursed and widely available, consistent with the principles of patient safety, market-based policies, and value for patients.*

CMS plans to identify the qualifying devices by the definition of breakthrough devices pursuant to Section 3051 of the 21st Century Cures Act, a definition that essentially mirrors that of breakthrough drugs and biologics.

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[1] Indications for Hematopoietic Cell Transplant and Immune Effector Cell Therapy: Guidelines from the American Society for Transplantation and Cellular Therapy. Biology of Blood and Marrow Transplantation, March 9, 2020.
[2] CMS MLN Matters: Billing Instructions for Beneficiaries Enrolled in Medicare Advantage (MA) Plans for Services Covered by Decision Memo CAG-00451N. Issued October 24, 2019.
[3] CMS.gov; Formal Request for National Coverage Determination for Chimeric Antigen Receptor T-cell Therapies, Submitted by UnitedHealthcare on February 22, 2018.

> *Breakthrough devices are those that (1) that provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating human disease or conditions; and*
> *(2) (A) that represent breakthrough technologies;*
> *(B) for which no approved or cleared alternatives exist;*
> *(C) that offer significant advantages over existing approved or cleared alternatives, including the potential, compared to existing approved alternatives, to reduce or eliminate the need for hospitalization, improve patient quality of life, facilitate patients' ability to manage their own care (such as through self-directed personal assistance), or establish long-term clinical efficiencies; or*
> *(D) the availability of which is in the best interest of patients.*

The FDA utilizes the definition of breakthrough therapy codified by Section 902 of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA):

> *A breakthrough therapy is a drug:*
>
> - *intended alone or in combination with one or more other drugs to treat a serious or life threatening disease or condition and*
> - *preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.*

Given the similarity between the designations, CMS should consider broader efforts to clarify coverage for breakthrough drugs and biologics as they are approved to increase the likelihood of ensuring beneficiary access, per CMS' stated goal.

The process CMS outlines for post-interim/permanent coverage needs additional clarification to support long-term access.

CMS proposed an automatic four-year period of initial coverage for devices that opt-in to the MCIT program. CMS indicates at the end of the initial coverage period, the device would become subject to either NCD or LCD, with consideration of opening an NCD at the end of the six months if no LCDs have been established. If the MCIT coverage expires and there is a void of LCDs or an NCD for an extended period of time, there will almost definitely be a period of coverage status and access confusion, as coverage will revert to claim-by-claim adjudication, identical to what MCIT intends to avoid at the time of product approval. Additionally, beneficiaries may be asked to sign a Hospital-Issued Notice of Non-Coverage (HINN), Advanced Beneficiary Notice and/or provide a deposit for access to these breakthrough technologies during this time of uncertainty, particularly if they are high-cost items and services. This places undue financial and administrative burdens on the beneficiary and their family while already navigating a difficult clinical situation and health status, and also further exasperates disparities in access to care for those with limited financial resources.

If CMS intends for the post-interim period to result in national coverage, whether by uniform LCD or NCD, it needs to instruct the MACs or stand ready to issue a coverage or non-coverage determination by the end of the four year initial period so as not to open a period of uncertain access for beneficiaries.

Additionally, manufacturers should be required to lay out data-collection plans informed by any concerns noted by the CMS Coverage and Analysis Group and/or MACs at the time interim coverage is granted. If CMS anticipates CED to be likely, it should pursue a study plan in conjunction with the manufacturer and affected provider groups that is aimed at resolution by the end of the interim coverage period. If CMS initiates an NCA at the end of the interim coverage cycle, as opposed to working towards a decision during the interim period, it may be introducing multiple periods of uncertainty – the first during the initial 9-12 months of a typical NCA cycle and, if CED was deemed necessary, during the CED study development and opening time period in addition to the reconsideration cycle at the completion of the CED study.

While LCDs may appear to be the less burdensome route, MACs lack the up-to-date medical knowledge to adequately consider what items and services should be covered as the field evolves – as our providers have experience regularly with an evolving and innovative field like HCT. It is highly likely that MACs will be incapable of keeping pace with the rapidly changing medical environment in which discoveries occur on a daily basis. Expertise in medical science should rest within the national level CMS teams, rather than being delegated to MAC level entities with varying knowledge bases. Further, we encourage CMS and FDA to work to identify additional opportunities for collaboration and discussion to minimize the need for potentially duplicative work across Agencies.

Additionally, the situation is complicated for the many providers that have more than one MAC. How does CMS envision the process when different MACs issue conflicting decisions about life-saving coverage, particularly in the face of clear and supportive clinical evidence? As an example, coverage for allogeneic stem cell transplant necessary to treat certain types of otherwise fatal lymphoma is granted by some, but not all, MACs and the governing NCD is silent. This leaves beneficiaries with the terrible options of deciding not to pursue care or seeking a center willing to take a financial risk on their treatment, while knowing they may end up being ultimately financially responsible. Additionally, we note that Medicare Advantage (MA) plans are required to comply with NCDs, but not with Local Coverage Decisions (LCDs). Hence, greater reliance on LCDs over NCDs would eliminate the guaranteed coverage for beneficiaries in MA plans, which is an increasing proportion of beneficiaries. MA Plans may then have a legally justifiable reason for not offering beneficiaries the same innovative care that is available to their commercially insured populations. Beneficiaries do not anticipate needing this type of specialized care in advance of making their Medicare coverage selections, nor do they understand how the coverage requirements differ across the various coverage options, leaving them vulnerable to being without access to the care they need at the time they need it.

Finally, as stated previously, we anticipate that MA plans will initiate NCA requests due to their interests in offsetting the costs of new technologies to FFS Medicare, as they continue to not likely

be included in prior year MA bids and subsequent contracts. If an NCD is issued, the covered item or service is evaluated against the Significant Cost Criterion and the outcome impacts the party responsible for covering the costs of the item or service. **CMS should consider how this process will be handled in the context of MCIT implementation, if finalized.**

The ASTCT does not support the codification of "Reasonable and Necessary" as proposed in the rule.

We disagree that the Executive Order requires that CMS codify the definition of "reasonable and necessary," already in statute, through the regulatory process. Executive Order 13890 instructed CMS to encourage innovation by:

> *(ii) clarifying the application of coverage standards, including the evidence standards CMS uses in applying its reasonable-and-necessary standard, the standards for deciding appeals of coverage decisions, and the prioritization and timeline for each National Coverage Determination process in light of changes made to local coverage determination processes;*

ASTCT requests that CMS focus on these processes in light of the access challenges outlined earlier in this letter. CMS could clarify the application of coverage standards by indicating when it intends to allow for the opening of NCA, such as in the case of the unusual analysis opened for CAR-T, an FDA-approved biologic, which was a category previously not subject to the NCD process. The CAR-T NCA introduced a 15-month period of coverage uncertainty and greater provider burden as ASTCT engaged our members in responses, calls and participation in the MEDCAC meeting, resulting with an NCD that simply covers CAR-T to the FDA label. Repeating this process for each new approval will not be sustainable for either CMS or the affected provider communities.

CMS could also seek comment on the types of evidence standards it currently employs in coverage decisions to allow for a broader discussion of the use of real-world evidence, consensus practice guidelines and registry data to support both claim-by-claim adjudication and national and local coverage determinations.

Our concerns with utilizing commercial payer policies as benchmarks for coverage are multiple, including access to proprietary payer documentation, vast heterogeneity among policies by payer size, region and type, and a lack of subject matter expertise in subspecialty areas – **the primary clinical space in which breakthrough therapies are focused -** by commercial payer medical policy staff. Additionally, commercial payers often limit clinical eligibility further than the FDA label, and they may also require specific sites of care particular to their local network and region, or include step therapy requirements. If CMS is looking to an external reference for understanding the value of new therapies, we suggest consideration of clinical practice guidelines that are maintained by professional medical societies, such as the ASTCT's Practice

Guidelines.[4] CMS could request that the relevant society review new practices or therapies for integration and share the guidelines with CMS and MACs for reference use.

While we understand the resource constraints that CMS is facing, we do not think the deference to commercial policies will resolve them; in fact, the process of identifying relevant policies, conducting a thorough review, analyzing and resolving the noted conflicts between policies will be both a significant amount of work for the MACs and the type of work that they are not currently staffed to perform. Commercial policies typically undergo annual reviews and updates, which would likely necessitates the same process on the MAC side in order to understand how the modifications and new evidence in the field may need to be integrated into the reference documents generated that reference these commercial policies. Finally, our assumption is that MACs would need to identify which specific commercial payer policies would be utilized as references for the purposes of making the reasonable and necessary determination, allow for public comment, and issue final justification documentation for providers to reference in individual cases. Repeating this administrative process of multiple breakthrough devices or biologics each year would be a significant additional burden on both MAC staff and the affected provider specialty societies.

As stated earlier, the NCA process is lengthy and creates an extended period of coverage uncertainty during which MACs are able to cite the NCA process itself as a reason to not cover the item or service being evaluated. While the timeline may be difficult to trim further due to public comment periods, we request that CMS clarify that the MACs continue to cover the item or service under consideration until such a point that a final determination is made. Many of the items and services that warrant an NCA, such as HCT or CAR-T, are not optional for the beneficiaries in need of life-saving intervention and many are not likely to survive a minimum 9-to-12 month wait for the process to be concluded.

If CMS is considering opening an NCA for a new item or service, we strongly encourage CMS to reach out to the relevant specialty society for a discussion with the group's clinical experts. These initial discussions may allow for CMS to better understand if an NCA is truly needed and, if so, what specific clinical questions should be the focus of the inquiry and how best to structure the initial proposed decision. These upfront conversations could dramatically modify the timeline and trajectory of the NCA process, allowing for mutually beneficial efficiencies.

[4] ASTCT Clinical Practice Guidelines: https://www.astct.org/learn/practice-guidelines

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The ASTCT wishes to express its appreciation for the opportunity to provide these comments on the MCIT Proposed Rule. The ASTCT welcomes the opportunity to discuss these recommendations in more detail or to answer any questions you may have. Please contact Alycia Maloney, ASTCT Director of Government Relations, at amaloney@astct.org for any follow up issues.

Sincerely,

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