March 16, 2019

Ms. Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building
Room 445-G
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Subject: National Coverage Analysis for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancers (CAG-00451N)

Dear Administrator Verma, Ms. Jensen and Drs. Szarama and Paserchia:

The undersigned organizations represent hundreds of thousands of cancer patient and survivors. With these constituents in mind, we appreciate the opportunity to provide comments on the recently-released National Coverage Analysis for Chimeric Antigen Receptor (CAR) T-cell therapy for the treatment of cancers.

We gratefully acknowledge the effort that the Centers for Medicare & Medicaid Services (CMS) has committed to understanding the impact of CAR T-cell therapy on people with cancer. However, the agency has proposed a coverage with evidence development (CED) process that rejects the principle that U.S. Food and Drug Administration (FDA) approval (a finding that a drug is safe and effective) is adequate to support a finding that a drug is reasonable and necessary and will be covered by Medicare. This is a change from drug coverage policy that may have significant effects on patient access. We ask CMS for clarification of its decision to reject usual coverage principles and to explain how patient access will be protected during the CED process.

The two CAR T-cell therapies which have been approved by the FDA impact patient populations which experience poor outcomes and have limited treatment options; in some cases, no other treatment options exist. In addition to these two treatments, there are more than 250 clinical trials currently studying the use of cellular therapies.

To accelerate access to effective, innovative therapies and collect the data necessary to aid in the delivery of quality cancer care to beneficiaries, CMS must clarify patient eligibility for the CED process. We recommend that coverage not be limited to relapsed and refractory cancers but instead be consistent with FDA-approved indications. We also urge that CMS consider how products that may be approved during the course of this CED process be included.

Similarly, the provision which allows CAR T-cell therapy to be administered only to individuals that have "not currently been experiencing any comorbidity that would otherwise preclude patient benefit" lacks precision. The proposal does not make clear how CMS will define these comorbidities nor which party will be responsible for confirming and monitoring their existence. The patient population currently treated with these therapies is very ill and it is likely that some will suffer from other chronic conditions. To protect the integrity of the patient-doctor relationship and account for the evolving use of CAR T-cell therapy across different disease states, histologies and patient populations, only physicians should determine whether their patient is able to benefit from the therapy. We also believe that additional guidance around "new primary cancer diagnosis" and "the use of more than one therapeutic dose of a specific CAR T-cell product" is necessary. Here too we believe that a patient's healthcare provider is in the best position possible to determine when and whether a patient will benefit from CAR T-cell therapy and should not be limited by narrow coverage policy.

As the use of CAR T-cell therapy expands, it is clear that CMS seeks to better understand the use of this treatment; its ability to improve patient survival; and patients' experience with treatment. To ensure that any data collection mechanism succeeds in achieving these goals it is critical that the process and means by which the data will be gathered and aggregated is unambiguous. This includes the process which CMS will use to approve studies and registries that qualify the coverage standards. No standards have been outlined which address whether the questions and requisite data are appropriate, nor the process by which disease-specific experts may be consulted to ensure that questions are suitable to a specific patient population. It is also necessary to define the settings in which therapy is administered - which may change during a single course of treatment - and the factors which qualify an institution and its staff to administer CAR T-cell therapies.

The utility of collecting patient-reported outcomes (PRO) to complement other cancer treatment data is well demonstrated and we recognize CMS for considering the patient perspective. If implemented, consistency of data collection through a single PRO tool is recommended to avoid confusion and guarantee a robust body of data. Acknowledging the functional and health status of the patients currently treated with CAR T-cell therapy is also necessary. Because these patients are severely ill, we recommend that CMS account for patients' ability and election to participate in any data collection exercise. Coverage should not be jeopardized for those patients who cannot or will not participate. Toward that end, greater clarity is also needed to understand which party will be

responsible for patient education and tracking the patient and their participation in required data collection activity.

Per federal regulation, National Coverage Decisions (NCD) become effective upon the date of their final publication. In the case of the NCD for CAR T-cell Therapy for Cancers, that date is projected to be May 17, 2019. Given the length of time and complexity of CAR T-cell therapy administration, it is likely that a single date is an unreasonable effective date for this NCD. It is important for CMS to take into consideration the length of time typically required for the entire treatment process to take place and specifically outline its plan to ensure that no patient or hospital risks non-coverage during the NCD's announcement and implementation. Blood cancer patients currently treated with CAR T-cell therapy cannot afford the time delay which may accompany a lack of clarity in coverage. Such clarification will ensure that patients suffering from life-threatening cancers will have access to this treatment, as recommended by their physicians, without interruption.

We appreciate this opportunity to provide comments to CMS and remain eager to work together to ensure that beneficiaries' access to this innovative and potentially life-saving therapy is not compromised.

Sincerely,

American Cancer Society Cancer Action Network, Inc.

Blood & Marrow Transplant Information Network

Cancer*Care*

Cutaneous Lymphoma Foundation

International Myeloma Foundation

International Waldenstrom's Macroglobulinemia Foundation

Lymphoma Research Foundation