June 15, 2018

Tamara Syrek Jensen, JD
Director
Evidence and Analysis Group
Centers for Medicare &
Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Katherine B. Szarama, PhD
Lead Analyst
Evidence and Analysis Group
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Lori A. Paserchia, MD
Lead Medical Officer
Evidence and Analysis Group
Centers for Medicare &
Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

Dear Ms. Syrek Jensen and Drs. Szarama and Paserchia:

The Association of American Cancer Institutes (AACI) represents 98 National Cancer Institute (NCI)-designated and academic-based cancer research programs in the U.S. and Canada that excel in cancer research, clinical care and education. AACI cancer centers and their regional community-based networks, provide care for an estimated 700,000 newly diagnosed patients with cancer, representing as much as 40 percent of the nation's cancer burden. Cancer therapies have improved dramatically over the last several years, due to the development and discovery of novel therapies resulting from the science and research conducted at the AACI cancer centers.

AACI appreciates the opportunity to submit comments to the Centers for Medicare & Medicaid Services (CMS) regarding the decision to examine coverage for chimeric antigen receptor T-cell (CAR T) therapies. CAR T therapies represent a critical advancement in treating malignancies, and evidence shows these therapies provide important clinical benefits to patients. Specifically, these therapies provide innovative ways to treat cancer by genetically reprogramming the patient's white blood cells to attach to their tumor cells and induce cancer cell death, resulting in significant increases in complete remission and survival for patients compared to what would have been expected with conventional chemotherapy.

In August of 2017, CAR T immunotherapy tisagenlecleucel (Kymriah®) was approved by the Food and Drug Administration (FDA) for the treatment of pediatric and young adult acute lymphoblastic leukemia. The price for the one-time infusion is $475,000. In October of 2017, the FDA approved a second product, axicabtagene ciloleucel (YESCARTA®) for adult patients with certain types of large B-cell lymphoma. The cost of the YESCARTA® product has been widely published as $373,000 per treatment. On May 1, a

second approval for CAR T was granted to expand the Kymriah® treatment to adults with relapsed large B-cell lymphoma.

CAR T therapies are encouraging, yet the reimbursement barriers associated with CAR T are apparent. The cost for patient infusion ranges from $350,000-$500,000. This may not include additional costs associated with the preparation and administration of the therapies. This also does not consider instances where patients must be hospitalized, or monitored closely to ensure the side effects a patient incurs are not life- threatening.

Nearly half of AACI's cancer centers are approved to offer CAR T therapies to qualified patients. For some patients with no available alternatives, the therapies have displayed complete and effective responses. The potential for these therapies, and those which come after them, to provide patients with treatments that will ultimately save and prolong their life is a beacon of hope for the oncology community.

AACI cancer centers have expressed concerns about the FDA approving the CAR T drugs without ensuring billing and reimbursement models were in place with CMS and other major payors. Due to the restrictions and requirements necessary to safely administer these products, most AACI cancer centers are taking on a disproportionate financial risk because the care treatment sites are found in large patient catchment areas. The high costs for providing these therapies, along with low reimbursement, contribute to creating significant financial risk for AACI cancer centers and could result in limitations centers' ability to offer CAR T therapies to Medicare patients.

AACI was disappointed to learn that CMS would open the national coverage decision (NCD) process to determine whether therapies are medically accepted treatments for on-label use. A NCD is not needed for coverage of FDA-approved therapies. The initiation of the NCD process creates uncertainty surrounding reimbursement for Medicare beneficiaries and may cause barriers for the patient. This barrier may lessen a patient's chance to receive these novel therapies in a timely manner. Because YESCARTA® and Kymriah® are FDA-approved anti-cancer drugs, AACI supports consistent national coverage. AACI therefore requests that CMS issue clarification to providers, Medicare Administrative Contractors (MACs) and Medicare Advantage Plans (MA Plans) noting that YESCARTA® and Kymriah® are covered for their medically-accepted use.

CMS states in the national coverage analysis that, "initial studies were also confined to the inpatient hospital setting." While the manufacturer did initially restrict provision of YESCARTA® during its clinical trials, the current package insert does not restrict provision to the inpatient setting. AACI cancer centers anticipate as these therapies develop and institutions adapt to administering these therapies that CAR-T administration will shift to the outpatient care setting. Physicians will require flexibility in the ability to offer such therapies, as indications and/or product safety profiles change. The ability to choose these therapies should be left to the patient and physician. CMS should not issue a NCD to study or limit site of care appropriateness.

Additionally, AACI considers the CMS inclusion of clinical services with the payment for delivery of a drug to be in opposition to previously established CMS-instructed standard provider billing guidance and practices. The inclusion of clinical services with the payment for delivery of the drug results in a bundled care scenario. If bundled care payment is the intention of CMS, AACI requests that a proposal be made through the Outpatient Prospective Payment System (OPPS) rule-making process to allow for full stakeholder engagement and commentary. AACI has established a CAR T Working Group comprised of

individuals at AACI cancer centers charged with administering and paying for these therapies. We would be happy to serve as advisors, or provide advice as further decisions are made by CMS.

Reimbursement issues are also prominent with commercial payors as there is no established industry payment methodology for new CAR T therapies. Some cancer centers are negotiating single-case agreements (SCA) for their patients who require access to CAR T. However, the SCA's do not cover the full cost of therapy. AACI foresees that these therapies will evolve and become the standard of care for cancer. As these therapies become more successful and develop further, SCA negotiation will become increasingly burdensome for cancer centers. When a patient is faced with a cancer diagnosis, and is seeking a therapy that has the potential to save their life, ensuring coverage of the therapy should be of paramount concern to CMS and other payors.

We respectfully ask that CMS consider:

- Providing consistent national coverage for CAR T therapies;
- Issuing clarification to providers, MACs, and MA Plans noting YESCARTA® and Kymriah® are covered for medically-acceptable uses;
- Allowing for flexibility and ability for patient and physician to choose medically necessary treatment;
- Engaging stakeholders if bundling of clinical services with payment for the delivery of the drug is CMS' intention; and
- Involving cancer centers and other relevant stakeholders in the CAR T evaluation process.

As these therapies expand and develop further, it is important for CMS to ensure that all patients can access these therapies, if recommended by their caregiver. AACI requests that CMS maintain its commitment to allowing cancer centers to practice medicine. This will allow for treatment decisions to remain with the patient and their physician.

Thank you for considering our views.

Sincerely,

Stanton L. Gerson, MD
AACI President

Roy A. Jensen, MD
AACI Vice President/President-Elect

Barbara Duffy Stewart, MPH
AACI Executive Director