



February 21, 2020

The Honorable Alex Azar
Secretary
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, D.C. 20201

The Honorable Seema Verma
Administrator
Centers for Medicare and Medicaid Services
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, DC 20201

The Honorable Mick Mulvaney
Director
Office of Management and Budget
725 17th Street NW
Washington, DC 20503

Dear Secretary Azar, Administrator Verma, and Director Mulvaney:

The American Society for Transplantation and Cellular Therapy (ASTCT) and the American Society of Hematology (ASH) appreciate the continued engagement of the Department of Health and Human Services (HHS) and the Centers for Medicare & Medicaid Services (CMS) with our Societies regarding Chimeric Antigen Receptor T-cell (CAR-T) therapy. We are writing today because it is critical that we work with HHS, CMS, and the Office of Management and Budget (OMB) to develop a sustainable and equitable payment mechanism for CAR-T therapy that can be implemented in the fiscal year (FY) 2021 Inpatient Prospective Payment System (IPPS).

The ASTCT is a professional membership association of more than 2,200 physicians, scientists, and other healthcare 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 current Food and Drug Administration (FDA) approvals for CAR-T therapy.

ASH represents more than 18,000 clinicians and scientists worldwide who are committed to the study and treatment of blood and blood-related diseases. These disorders encompass malignant hematologic disorders such as leukemia, lymphoma, and multiple myeloma, as well as non-malignant conditions such as sickle cell anemia, thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. In addition, hematologists are pioneers in demonstrating the potential of treating various hematologic diseases and continue to be innovators in the field of stem cell biology, regenerative medicine, transfusion medicine, and gene therapy.

Equitable payment under IPPS is vital for institutions administering CAR-T therapy as the inpatient setting remains the most clinically appropriate place to administer the currently approved CAR-T products (Yescarta and Kymriah) because of the potential for severe, adverse side effects. As we shared in our letter to Administrator Verma dated November 1, 2019, our Societies fundamentally see cellular and gene therapies as ushering in a new era of medicine, one that CMS' current IPPS could never have

anticipated – CAR-T therapy is the first of this class. All agencies, HHS, CMS, and OMB, must work together to balance protecting patient access to care with responsible stewardship of the Medicare trust fund. The Medicare program must adequately address the costs of treating patients with CAR-T therapies in FY 2021 to improve patient access to care and not risk stifling innovation of future CARs and other cellular and gene therapies.

Unfortunately, the current Medicare inpatient payment system does not adequately reimburse centers providing CAR-T therapy. CMS approved a new technology add-on payment (NTAP) for CAR-T therapy and increased the maximum NTAP from 50 to 65 percent of the product cost in the FY 2020 IPSS rule for a total of \$242,450. Additionally, CAR-T cases are currently classified in MS-DRG 016 (Autologous Bone Marrow Transplant with CC/MCC or T-Cell Immunotherapy) with an average reimbursement of \$43,000. Even with hospital specific adjustments, almost all the centers providing access to this life saving therapy lose money on each Medicare CAR-T beneficiary. As a result, some certified centers are choosing not to provide this life-saving treatment given significant financial losses even with the NTAP in place. Any future payment policy that erodes reimbursement will further limit patient access.

We have estimated that if standard rate-setting methodology is used to create a new Medicare Severity Diagnosis Related Group (MS-DRG), hospitals would, at best, be reimbursed approximately \$131,000 prior to adjustments being applied for the entire care episode. This would represent just over 35 percent of the actual product acquisition costs and would further exacerbate the current financial impact on institutions and do little to address patient access issues to CAR-T therapy.

FY 2021 is a pivotal year for inpatient CAR-T therapy payment policy. The decisions being made now will have significant consequences, not only for the two currently approved CAR-T products, but also for more than 900 cellular and gene therapy products in development. Therefore, ASTCT and ASH urge the Administration to take bold action in the FY 2021 IPSS proposed rule and have made the following recommendations to CMS:

- Maintain NTAP for CAR-T therapy through FY 2021; or
- If NTAP is not maintained:
 - Create a new MS-DRG for T-cell immunotherapy using only FY 2019 CAR-T claims with no clinical trial Z00.6 diagnosis code and with pharmacy charges greater than \$373,000; and
 - Use a pharmacy off-set similar to CMS' existing device off-set mechanism to pay for the T-cell immunotherapy MS-DRG for CAR-T claims where the hospital receives the cell therapy product at no cost.

We recognize that both options would be precedent setting, but they would provide the Administration with the time needed to develop a sustainable and equitable reimbursement policy for these innovative therapies.

Concerns with Data

As we have previously stated, the limitations of the data currently available for rate-setting would make it difficult for CMS to establish an equitable relative weight for a new CAR-T MS-DRG using the existing

rate-setting methodology. Using the FY 2019 claims data to establish a new autologous T-cell immunotherapy MS-DRG would have the following characteristics not typical of data usually used for rate-setting:

- Lower volume of cases than expected;
- A large proportion of clinical trial cases compared to other MS-DRGs;
- No visibility into the CAR-T therapy product charge separate from all other pharmacy charges for the full fiscal year because revenue code 0891 (Special Processed Drugs – FDA (Food and Drug Administration) Approved Cell Therapy - Charges for Modified cell therapy) was not available until April 1, 2019;
- And high variability in pharmacy charges.

The Societies believe we will begin to see improvements in these areas in time to develop a reasonable MS-DRG in FY 2022.

Maintain NTAP for CAR-T Therapy through FY 2021

Based on our concerns with the data, ASTCT and ASH have requested that CMS maintain the current NTAP for the FDA-approved CAR-T therapy products for FY 2021 and delay creating a new MS-DRG until FY 2022. We believe this is the only policy that will maintain appropriate patient access to this therapy in the short term while allowing CMS to carefully consider how to develop an equitable relative weight for the MS-DRG for CAR-T therapy that will set a precedent for future cellular therapies.

NTAP payments for CAR-T therapy have only been available for two fiscal years, yet CMS recognizes services as new “two to three years from the date of establishment” in 42 CFR §412.87(b)(2). We have asked that CMS provide the NTAP payment for the two FDA-approved CAR-T products for a third year and use this time to collect better data for rate-setting. Ultimately, we believe CMS, patients, and providers will benefit from this extension that allows the Agency to take the additional time to collect accurate data and evaluate if and how its IPPS mechanisms should be modified to address this new and unique class of therapies.

Create a New MS-DRG

Should the Administration choose not to extend the NTAP for an additional year, we respectfully request that CMS implement specific methodologies when developing a new MS-DRG for CAR-T therapy to minimize the deficiencies in the data. Specifically, we ask CMS to set a new precedent as outlined above from its usual rate-setting methodology. We believe these changes are consistent with CMS’ general authority under sections 1886(d)(4)(B) and (C) of the Social Security Act to create a new MS-DRG that allows CMS to assign and update appropriate weighting factors in a manner that reflects the resources involved with immune effector cell therapy, including drug acquisition costs.

We believe this approach will improve access to transformative therapies by Medicare beneficiaries’ long term. Developing an appropriately reimbursed new T-cell immunotherapy MS-DRG will require CMS to grapple with the dual issues of 1) having a very large percentage of clinical trial cases making up the MS-DRG total case volume and 2) a clinical care episode in which the new technology’s cost constitutes an extreme proportion of the total case cost. We do not see record of CMS ever addressing these issues simultaneously.

We simulated what the FY 2021 payment rate for a new T-cell immunotherapy MS-DRG would be using all CAR-T cases from the first two quarters of FY 2019 SAF data and CMS' usual rate-setting methodology, including trimming, and the result is a national unadjusted payment of approximately \$131,000 (based on FY 2020 standardized national payment from the final rule correction notice) excluding possible outlier payment. This will result in a significant under-payment given that each provider is required to pay \$373,000 for acquisition of the drug for diffuse large B-cell lymphoma (DLBCL) before providing any additional clinical services. This does not represent fair and equitable payment, nor would it be consistent with CMS' general authority under sections 1886(d)(4)(B) and (C) of the Social Security Act.

Given the large number of therapies in the pipeline, we believe it is absolutely critical for CMS to act now, at the outset, to establish an appropriately reimbursing T-cell immunotherapy MS-DRG so that the clinicians and hospitals on the cutting edge of this treatment are able to continue treating patients.

The Societies look forward to continuing to work closely with this Administration to find the most equitable and sustainable solutions to reimburse for CAR-T to ensure appropriate patients have access to this therapy. For any questions please contact ASTCT's Director of Government Relations, Alycia Maloney, at amaloney@astct.org or ASH's Policy & Practice Manager Leslie Brady, at lbrady@hematology.org.

Sincerely,



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Stephanie J. Lee, MD, MPH
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cc: Stephen Hahn, MD, FDA Commissioner
Russell Vought, Acting OMB Director
Joe Grogan, Director, Domestic Policy Council