AMERICAN SOCIETY of GENE & CELL THERAPY

June 24, 2019

The Honorable Seema Verma, Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
P.O. Box 8011, Baltimore, MD 21244-1850

Attention: CMS-1716-P: Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and Long Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2020 Rates

Dear Administrator Verma:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to comment on CMS-1716-P. ASGCT is a professional membership organization representing over 3,000 individuals, including scientists, physicians, and other professionals in gene and cell therapy working in settings such as academic institutions, hospitals, and biotechnology and pharmaceutical companies. Many of our members have spent their careers in this field performing the underlying research that has led to today's robust pipeline of transformative therapies.

A core portion of the Society's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. ASGCT supports maximum reimbursement levels of approved therapies in order to foster patient access to these therapies. This position does not imply endorsement of any individual pricing decisions.

The level that Medicare reimburses for CAR T-cell therapy through current mechanisms often leaves a significant gap in payment to certified hospitals compared to their combined costs for services and for the biologic therapy, and ASGCT is concerned that such losses may be unsustainable for current providers. In addition, some hospitals that are qualified and had planned to become authorized treatment centers have not yet started providing CAR T-cell therapy due to the high risk for substantial financial losses. This situation poses potential barriers to Medicare beneficiary access to these therapies by decreasing the already limited number of prospective authorized treatment centers, and potentially affecting the proximity of treatment to seriously ill cancer patients. ASGCT appreciates that CMS has acknowledged these concerns in the FY2020 IPPS proposed rule through its proposals and requests for comment.

Our comments will focus on three areas of significance to ASGCT members for patients who have the opportunity to benefit directly from access to CAR T-cell therapies for the treatment of certain instances of diffuse large B-cell lymphoma and acute lymphoblastic leukemia: increasing the new technology add-on payment (NTAP) cap in FY 2020 for PPS

hospitals; assignment of a new MS-DRG for CAR T-cell therapy; and payment alternatives for CAR T-cell therapy.

Increasing the NTAP cap for all new technologies

We appreciate CMS acknowledgment of the need to increase the NTAP cap. ASGCT agrees with the stakeholder feedback CMS has received since the new technology add-on payment was first established in 2001, that the 50-percent limit in the formula does not adequately reflect the costs of new technology, nor does it sufficiently support healthcare innovations. As a result, the Society recommends CMS change the 50-percent cap in its current NTAP formula to a much higher percentage. We appreciate that CMS proposed an increase to 65 percent but consider it insufficient and ask the Agency to increase this level as much as possible, ideally to 100 percent. The additional cost to CMS of increasing the NTAP cap should be manageable, since historically CMS has not utilized all allocated NTAP funds.

Increasing the NTAP cap should result in greater payment to providers who choose to code, bill, and charge in ways that allow them to obtain the maximum level. However, changing the NTAP cap for all NTAPs alone would insufficiently address continued high losses for providers of CAR T-cell therapy who decide they do not wish to utilize certain charging practices (see appendix). The Society wishes to support transparency in provider charging practices and greater equity in reimbursement levels to all providers for CAR T-cell therapies, and therefore proposes an additional mechanism to do so below.

CMS stated in the September 7, 2001 final rule (66 FR 46919) that it does not believe it is appropriate to pay an add-on amount equal to 100 percent of the costs of new technology because there is no similar methodology to reduce payments for cost-saving technology. However, because NTAPs are temporary, for only two to three years, the increase in the NTAP cap would likely have a minimal effect on limiting use of additional new, potentially cost-saving technologies that receive approval during the NTAP period.

Assignment of a new MS-DRG for CAR T-cell therapy

ASGCT supports the CMS proposal not to modify the current MS-DRG assignment for cases reporting CAR T-cell therapies for FY 2020 because of the limited number of cases with widely varying submitted charges. Although patients receiving CAR T-cell therapy differ from patients receiving autologous bone marrow transplantation, the Society agrees that CMS should continue to assign CAR-T cases to MS-DRG 016, versus creating a separate new MS-DRG for this therapy, until more comprehensive clinical and cost data is collected. For the same reason, we support the CMS proposal to continue new technology add-on payments for FY 2020 for the two CAR T-cell therapies that currently have FDA approval (KYMRIAH™ and YESCARTA™).

ASGCT foresees the need in future years to create a new MS-DRG specifically for cases involving CAR T-cell therapies, based on the availability of additional data that is more accurately and consistently reflective of actual costs to providers. We appreciate and support CMS's early consideration of identifying new and alternative methods for reimbursing the product cost of cell and gene therapies differently, evidenced in its inquiry on the advisability of using a percentage of the average sales price for reimbursement of the product cost. For this reason, while a future new MS-DRG could encompass the costs of the therapeutic product and hospital services provided to the patient, ASGCT recommends that a new MS-DRG, when assigned, reimburse for patient care costs alone, with a separate payment or MS-DRG group for the product. An approach that separates patient care service costs and product costs would allow the Agency to continue using the averaging process central to prospective payment systems

to pay for the patient care portion of the total case cost while allowing full visibility of the product cost, and allow CMS to use the same patient care MS-DRG for future innovation. It would also allow use of CMS's current application of hospital-specific adjustments for patient care but not for the product, which we believe is the most appropriate way for CMS to proceed. Significantly, it would also provide CMS the flexibility to employ value-based product payment models in Part A in the future.

Payment alternatives for CAR T-cell therapy

ASGCT welcomes CMS's requests for comments on providing additional levels of NTAP reimbursement and other payment alternatives for CAR T-cell therapies given their unique technology and benefit**.** We support altering reimbursement mechanisms to providers in a manner that takes into account how these groundbreaking therapies have altered treatment paradigms for beneficiaries.

The current maximum add-on payment of 50 percent, or even an increase as described earlier, does not allow for sufficient payment of CAR T-cell therapy, with costs that completely exceed the total MS-DRG but that provide extremely high value. The original limitation on the NTAP was intended to ensure that hospitals balanced the desirability of new technologies with the utility of standard of care treatment to avoid potential inappropriate use. ASGCT believes that exceptions should be made in cases in which standard of care treatment either does not exist at all or is ineffective, or the new treatment presents highly favorable patient outcomes. CAR T-cell therapy is unique in that it is an autologous, personalized cellular therapy product for indications for which standard of care outcomes are extremely poor.[i]

Eliminating the use of CCR in calculating the NTAP for CAR-T therapy

Removing the CCR in the NTAP calculation for CAR T-cell therapy by creating a uniform NTAP with a payment level of 65 percent would be an improvement in reimbursement levels but would still result in large losses for hospitals that apply lower levels of markup. Only at a uniform NTAP amount of 100 percent of product acquisition cost would NTAP payments be equal across all PPS hospitals, regardless of markup level. ASGCT prefers the solution described subsequently, which eliminates the need for high markups in order to obtain adequate reimbursement, provides increased transparency in provider charges, and collects data on actual product acquisition costs.

Utilizing CCR of 1.0 for CAR-T therapy (actual acquisition costs)

The Society wishes to clarify that the CCR of 1.0 concept for the CAR-T therapy would *not* entail inserting 1.0 into the formula for determining the calculated cost to be used in the NTAP and outlier calculations. Rather, this formula enables use of the actual acquisition costs, rather than inconsistent charges compressed by the order of magnitude of the overall hospital CCR.

The CCR of 1.0 concept is to utilize the following formula for determining calculated cost:

{[(Total inpatient charges on the CAR T-cell therapy claim) – (CAR T-cell product charge)] x (hospital overall CCR)} + (actual CAR-T product acquisition cost)

Therefore, applying this formula to a hypothetical claim in which total charges are $1,720,000 would result in the following calculated cost for a hospital with an overall CCR of 0.25:

$1,720,000 (inpatient charges) - $1,492,000 (CAR-T product charge) = $228,000 (patient service charges)

$228,000 (patient service charges) x 0.25 (hospital overall CCR) = $57,000 (hospital CCR-adjusted charges)

$57,000 (hospital CCR-adjusted charges) + $373,000 (actual product acquisition cost) = $430,000

This option, which uses the current formula to compute only the patient care cost by using CMS's charges-reduced-to-cost methodology, results in a more realistic calculated cost to providers for the provision of the therapy while also adding in actual upfront acquisition cost of the therapeutic product. Collecting actual acquisition costs can be done through the required use of the newly assigned value code 86. This methodology would also result in use of the outlier payment pool only in those cases in which patient care costs are in far excess of the overall payments, rather than allowing provider markup practices on the product cost to influence the outlier payment.

Recommendation

ASGCT recommends the following as the best option to both attain maximal reimbursement to providers and to equalize the reimbursement levels for providers regardless of charging practices:

- Increase the NTAP cap for all NTAPs from 50 percent to 100 percent
- For CAR T-cell therapy, determine the calculated cost for patient care services only through the current formula, and then add in the actual acquisition cost for the product before continuing with the NTAP and outlier calculations

Encouraging Value-Based Care and Lower Drug Prices

In the proposed rule, CMS invites comments on value-based care and lower drug prices. ASGCT supports such value-based contracting which ties a portion of product payment to the outcomes of the therapy, so that lower costs are incurred for less effective individual patient results. The best timing for implementation of voluntary value-based payment agreements between individual CAR-T manufacturers and CMS would be following the establishment of a new MS-DRG based on data collection of full acquisition costs in a greater number of patients than is currently available. ASGCT proposes that such arrangements be established initially on a voluntary basis to assess the impact of such arrangements on overall costs and patient outcomes.

In response to the CMS request for comment on how proposed payment alternatives affect incentives to encourage lower drug prices, ASGCT recommends consideration of the cost-effectiveness of CAR T-cell therapies compared to the costs of third and fourth lines of standard of care treatment for the current CAR T-eligible patient populations. The costs of standard of care treatment have not been adequately identified and may carry equal or higher costs for dismal patient outcomes.[i] Until such comparison data is available, ASGCT recommends creating wide availability to CAR T-cell therapy by decreasing large losses to providers to maximize patient access.

Other considerations

Future rate setting

The Society recommends that CMS require completion of the value code 86 field for collection of actual acquisition costs of CAR-T therapies to guide future rate setting of a new MS-DRG. If mechanisms other than the value code 86 are utilized to collect data for rate setting, removing clinical trial cases from the data in another manner would be important in establishing an accurate MS-DRG. Ideally, collection of value code data would be done for two years to collect sufficient accurate data for FY 2022 rate setting. Extending the CAR T-cell therapy NTAP through 2021 would facilitate this goal.

PPS-exempt centers

While these comments have focused on PPS centers, ASGCT has concern over appropriate reimbursement for all providers of CAR T-cell therapy to maximize patient access. Because PPS-exempt

centers treated a large proportion of the CAR T-cell therapy cases to date and will likely continue doing so, the Society requests that CMS also attend to the requests from PPS-exempt centers for facilitation of expedient relief from their reimbursement challenges to maintain patient access.

Conclusion

ASGCT appreciates the thoughtful consideration CMS is affording NTAP improvements for CAR T-cell therapy and to the NTAP system in general as science continues to produce innovative products that will revolutionize patient care. To summarize, ASGCT supports CMS implementation of the following options which we believe, combined, offer the most adequate reimbursement of, and therefore patient access to, CAR T-cell therapies:

- Change the NTAP cap for all NTAPs from 50 percent to 100 percent
- Determine calculated cost for patient care services only through the current formula, and add the actual CAR-T product acquisition cost, for purposes of computing the NTAP and outlier reimbursement
- Collect actual product acquisition cost data for CAR-T therapy through required use of value code 86
- Extend the NTAP for CAR T-cell therapy through 2021

We appreciate reimbursement decisions that reflect the changing and improved nature of new treatment technologies to encourage patient access to these potentially curative treatments. Please let us know if you have any questions for which we may be of guidance.

Sincerely,

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[1]Crump M, Neelapu SS, Farooq U, et al. Outcomes in refractory diffuse large B-cell lymphoma results from the international SCHOLAR-1 study. *Blood.* 2017;130(16):1800-1808.

Appendix

Hospital A: Sample Inpatient Hospital Claim			
FL 42 Revenue Code	FL 43 Description	FL 46 Units	FL 47 Total Charges
0121	Room & Board	14	$63,000
0250	Pharmacy	100	$45,000
0270	Supplies	20	$13,000
0300	Laboratory	520	$32,000
Other dept. charges	All other	50	$75,000
891	CAR-T product	1	$410,300
0001	Total Charges		$638,300
Note: Hospital A uses a 10% markup (rather than it's overall hospital cost-to-charge ratio of 0.25) to mark-up the $373,00 CAR-T product cost to $410,300			

Hospital B: Sample Inpatient Hospital Claim			
FL 42 Revenue Code	FL 43 Description	FL 46 Units	FL 47 Total Charges
0121	Room & Board	14	$63,000
0250	Pharmacy	100	$45,000
0270	Supplies	20	$13,000
0300	Laboratory	520	$32,000
Other dept. charges	All other	50	$75,000
891	CAR-T product	1	$1,492,000
0001	Total Charges		$1,720,000
Note: Hospital B does use its overall hospital cost-to-charge ratio of 0.25 (i.e., mark-up 400%) to mark-up the $373,000 CAR-T product cost to $1,492,000			

Claim Assumptions

(1) Same type of patient treated in both hospitals; and both hospitals are of a similar type

(2) Charges reflected are likely below average as there are no ICU days/stay or other complications being addressed

(3) The only difference between the two hospitals we are showing is their current FY 2018 mark-up practice

(4) Both hospitals have a wage-index of 1.0 and no adjustments for IME or DSH when it comes to calculating MS-DRG payments

	FY 2019	CMS' Proposal to Change the NTAP Cap	Our Version of CMS' Proposal to Change the NTAP Cap for all NTAPs	Our Version of CMS' Proposal to Change the NTAP Cap PLUS Operationalizing the Idea of a CCR of 1.0 Through Using Actual Product Acquisition Cost in the NTAP and Outlier Formulas for CAR-T Only	
Options [1]	Current	Change NTAP cap from 50% to 65%	Change NTAP cap from 50% to 100%	NTAP formula change applicable to all NTAPs as proposed by CMS from 50% to 65% AND use product acquisition cost in the NTAP and Outlier formula	NTAP formula change to 100% for CAR-T Only and using product acquisition cost in the NTAP and Outlier formula
NTAP	NTAP cap of 50% (product mark-up impacts whether max NTAP is received)	Change in NTAP cap is helpful but the product mark-up still impacts the amount of NTAP received		Change the NTAP calculation so that the CAR-T product charge is subtracted from total charges and then multiply by the hospital's overall cost-to-charge ratio to compute patient care cost; add to the patient cost what is reported in value code 86 for the CAR-T product cost and continue with the formula	
Outlier [2]	Current methodology (product mark-up impacts reimbursement)	Current methodology (product mark-up impacts reimbursement)		Change the outlier NTAP calculation so that the CAR-T product charge is subtracted from total charges and then multiply by the hospital's overall cost-to-charge ratio to compute patient care cost; add to the patient cost what is reported in value code 86 for the CAR-T product cost and continue with the formula	
Financial Impact for Hospital A: Reflects a 10% Mark Up	**($303,003)**	**($300,216)**	**($270,425)**	**($50,607)**	**($14,507)**
Financial Impact for Hospital B: Reflects a Four-fold Mark Up	**($61,325)**	**($50,607)**	**($14,507)**	**($50,607)**	**($14,507)**

Notes:

(1) Each option is based on the sample claims below, which have been shared with CMS by other stakeholders in the past. These are for illustration purposes only to show the impact of varying hospital charging practices on reimbursement given CMS' current formulas.

(2) The fixed loss outlier threshold for FY 2019 is $25,769 and proposed for FY 2020 is $26,994. The impact calculation uses a single outlier payment rather than separating out for operating and

Actual individual hospital financial impact will vary based on a hospital's charging practice, the hospital's own operating cost-to-charge ratio, applications of adjustments and a more detailed outlier calculation.