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

Dear Administrator Verma:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to comment on CMS-1735-P, the proposed rule for Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and Long Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2021 Rates.

ASGCT is a professional membership organization representing over 4,300 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 ASGCT's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. To that end, ASGCT supports Medicare payment policies that foster the adoption of, and patient access to, new therapies, and thereby encourage continued development of these innovative treatments. The Society's support of sufficient and appropriate reimbursement levels to facilitate patient access does not imply endorsement of any individual pricing decisions.

ASGCT is enthused to see CMS proposing to take action to address inadequate reimbursement for the administration of CAR T-cell therapy. CMS' decision to establish a new Medicare Severity-Diagnostic Related Group (MS-DRG) for purposes of the administration of CAR T-cell payments, determined without clinical trial cases, will contribute to more accurately reimbursing providers for the therapy and the cost of associated care after the new technology add-on payment expires. The Society sincerely appreciates CMS' proposed actions to do so.

ASGCT remains concerned about CMS' reimbursement mechanisms for future potentially life-saving therapies that could soon be available to Medicare

beneficiaries. Since 2017, stakeholders have watched CMS take great effort to incorporate CAR T-cell therapies within a payment system that was not designed to effectively document or reimburse expenses for transformative therapeutics with durable health outcomes.

ASGCT encourages CMS to consider a more holistic approach to new therapies that provides a clearer sense to all stakeholders—patients, providers, and manufacturers—regarding how Medicare will cover and pay for new cell and gene therapies coming to market.

In this comment letter, ASGCT provides a series of comments for Fiscal Year 2021, but also for future years. The key recommendations are as follows:

- ASGCT supports the proposal to create a new MS-DRG to reimburse for CAR T-cell therapy, and to exclude the cost of clinical trials from rate calculations.
- Beneficiaries and providers alike would benefit if CMS were to establish a consistent, predictable, and timely approach to incorporating new gene and cell therapies into the IPPS. This approach should include clear methods for addressing coding, new technology add-on payments (NTAPs), and DRG assignment, as well as timelines around decision-making that reflect the timelines associated with bringing a new therapy to market.

FY 2021 Proposal

New MS-DRG

ASGCT appreciates the thoughtful action CMS took in proposing MS-DRG 018 *Chimeric Antigen Receptor (CAR) T-cell Immunotherapy* for FY 2021. CMS' proposal to address the reimbursement for CAR-T administration demonstrates the Agency's commitment to providing beneficiaries with access to innovative therapies while maintaining long-term financial stewardship of the Medicare program.

Clinical Trials

ASGCT supports CMS' decision to exclude clinical trial cases from the payment calculations of MS-DRG 018. Given the relatively small number of cases available to CMS for the purposes of calculating MS-DRG weights, the importance of a case pool that accurately reflects the cost of the therapies is essential. To accomplish this goal, ASGCT supports the exclusion of cases with a clinical trial indicator (Z00.6) or with standardized drug charges below \$373,000 from the FY2021 rate-setting process.

We respectfully request additional clarity around whether CMS defines "drug charges" to include charges reported in revenue code 0891. Revenue code 0891 is a relatively new revenue code created by the National Uniform Billing Committee, called *Special Processed Drugs – FDA Approved Cell Therapy*. This revenue code went into effect as of April 1, 2019. In CMS' discussion of rate-setting for the proposed MS-DRG 018, CMS notes that "standardized drug charges of less than \$373,000" were utilized to exclude clinical trial claims from rate-setting. ASGCT is seeking clarification from CMS regarding whether the standardized drug charges referenced include charges submitted under revenue code 0891, not just those submitted under revenue codes 025x or 063x. If revenue code 0891 charges were excluded, ASGCT recommends CMS include them and recompute the relative weight for MS-DRG 018.

We are concerned by CMS' proposal to use the presence of Z00.6 to identify clinical trial cases moving forward. The Agency used a different definition of clinical trials (clinical trial indicator Z00.6 or standardized drug charges below \$373,000), as noted above for purposes of rate-setting. We believe a number of unintended payment consequences could result if CMS finalizes solely relying upon Z00.6 to identify clinical trial cases for purposes of reduced payment, including the potential for overpayment to a provider if the Z code is inadvertently omitted or if the biologic is provided as part of an Expanded Access Program. In addition, future clinical trials may utilize CAR T-cell therapy *for reasons other than the study of the investigational cellular product itself*. For example, a clinical trial focused on a specific drug's effects on complications of CAR T may be layered onto cases for which FDA-approved CAR-T products are being utilized. Due to the appropriate use of Z00.6 coding for this type of situation, these cases could result in reimbursement of only the reduced clinical trial MS-DRG 018 payment rate instead of the appropriate full MS-DRG 018 rate.

In each case, CMS could utilize the same "or less than \$373,000" requirement it is proposing for rate-setting in its payment logic for defining clinical trials for reduced payment in the future. CMS could also require use of value code 90 to indicate whether the provider incurred a cost for the cell therapy product. ASGCT recommends use of one of these methods to differentiate clinical trials cases moving forward.

Additional Considerations

ASGCT represents professionals dedicated to bringing life-altering new therapies to consumers by driving innovation in the gene and cell therapy industries. While the pace of scientific discovery ultimately shapes the pace of innovation, so too does the marketplace in which the therapies will arrive.

With the approval of tisagenlecleucel and axicabtagene ciloleucel three years ago, providers have assumed significant financial losses in order to provide these potentially life-saving therapies to Medicare beneficiaries. Previous rulemaking cycles brought some relief through higher NTAP caps but did not address the fundamental underlying shortcomings.

While CMS has answered the question of how it intends to pay for CAR T-cell therapy in 2021, it has not yet answered the question of how it will pay for additional therapies coming to market. For both additional CAR T-cell therapies likely coming to market imminently, and other innovative gene and cell therapy products in the pipeline, CMS does not provide a clear roadmap to reimbursement that will address the uncertainty of the last three years. As such, we offer the suggestions outlined below.

Coding – Future CAR T Products

We encourage CMS to develop a consistent, transparent, and timely approach to coding decisions for new products that minimizes uncertainty in the process. For example, we encourage CMS to approach coding for future CAR-T products in a way that will provide CMS product-specific data upon initial commercial use of a product to facilitate early data collection for rate-setting and to avoid potential gaps in reimbursement of new products that could arise from coding confusion. For these reasons, we respectfully recommend CMS clearly specify how new CAR-T therapies without product-specific codes are to be reported. An example of how to do so would be for CMS to utilize the existing non-specific new technology CAR-T XW0 ICD-10-PCS codes for all autologous CAR-T therapies, along with requiring

providers to report the NDC on inpatient claims to identify the specific drug product being utilized, for cell therapies reported with revenue code 0891.

New Technology Add-On Payments

We suggest that CMS take steps to make the NTAP more immediately accessible for new technologies coming to market, including altering the timing of consideration for those new products. The current system's tie to the annual rulemaking process is of particular concern. Timing for approval from the Food and Drug Administration (FDA) is based upon time of application filing and not an annual cycle. The NTAP process may be reformed to match a product's marketing approval, not an annual rulemaking cycle. CMS acknowledged the value of a more flexible approval process last year for certain antimicrobial products.¹

ASGCT therefore requests that CMS consider modifying the current NTAP process to allow for quarterly review of NTAP-qualifying products approved by the FDA, regardless of the approval pathway. The current NTAP process window (i.e., FDA approval requirement of July 1) is much too narrow, as CMS has already recognized for certain antimicrobial, antibacterial, and antifungal products. Doing so would allow manufacturers to apply for NTAP when they have data to complete an NTAP application and "pend" those applications deemed to meet the applicable NTAP criterion until the FDA has approved the product, and manufacturers have had an opportunity to bring the products to market.

Establishment of New MS-DRGs

Because the process for establishing new MS-DRGs is dependent upon CMS having sufficient cost and clinical data for a therapy, the creation of DRGs for treatments with small populations could end up being delayed well past the NTAP period. If CMS intends to pay for future gene and cell therapies in a similar fashion to CAR T-cell therapy, through NTAP assignment followed by the establishment of new DRGs, as applicable, we recommend that CMS continue to recognize the limited patient populations (especially for products indicated for rare diseases) when considering the number of cases sufficient to establish a new DRG. ASGCT commends the Agency for taking this factor into consideration for CAR T-cell therapy and encourages CMS to similarly establish DRGs for additional gene and cell therapies that qualify for separate DRGs prior to NTAP expiration to prevent patient access challenges.

Charge Compression

The issue of charge compression creates an added layer of uncertainty in the CAR T-cell market. CMS employs cost-to-charge ratios (CCRs) under the assumption that hospitals will mark up the cost of their charges. Yet CMS policies around price transparency push incentives in the opposite direction, leaving many hospitals with a difficult decision—to either appear to be inflating the cost of products or to be exposed to the negative financial impact of a CCR.

To create a more fair and balanced system that does not have troublesome incentives for providers or negative impacts on patient access to high-cost, single administration transformative gene and cell therapies, we respectfully request that CMS create a new national cost center specific to capturing

¹ Federal Food Drug and Cosmetic Act Section 506(h), 42 CFR 412.87(d)

charges for FDA-approved cell therapies reported using revenue code 0891. This will allow CMS to create a more robust and accurate CCR that would be used in future rate-setting to reduce revenue code 0891 billed charges to costs with significantly mitigated charge compression. This modification is especially important to setting the most accurate relative weight possible, which will be particularly important to those treatment centers in low wage-index areas, as they will not receive an upward adjustment through the application of the wage-index adjustment to help offset acquisition costs.

Alternative Payment Models

Lastly, ASGCT asks CMS to consider invoking the authority of the Center for Medicare and Medicaid Innovation (CMMI) to establish new, alternative payment models for gene and cell therapies, outside of the constraints of the IPPS. The uncertainty and confusion associated with the CAR T-cell experience had a significant impact on patients, providers, and manufacturers alike. Absent further reforms, the process could repeat itself with the next innovative product coming to market.

In CMS-2482-P, CMS demonstrates its willingness to facilitate innovative, value-based arrangements for products offered in the Medicaid program, which ASGCT commends. Specifically, CMS is proposing a series of rule modifications surrounding best price reporting that are expected to address stakeholder concerns about the current regulatory framework.

CMS has the tools necessary to explore comparable options for the Medicare program. ASGCT encourages CMS to prioritize that work, beginning with a request for information (RFI) that would allow stakeholders to provide the Agency with critical parameters for that work.

Conclusion

ASGCT applauds CMS for the proposed updates to Medicare payment policy for CAR T-cell therapy. Specifically, the proposal to establish a new MS-DRG and exclude clinical trial cases from the payment calculation is a timely and welcome update.

However, ASGCT continues to have concerns about the health and stability of the marketplace CMS has established within the Medicare program for other gene and cell therapies. Scientific breakthroughs are only helpful to patients who are able to access them, and the experience of CAR T-cell therapy has left our members concerned about the ability of current Medicare payment mechanisms to facilitate maximal, expedient access.

We welcome the opportunity to engage with CMS further on any of these issues and deeply appreciate this opportunity to provide our comments to the Agency.

Sincerely,



Stephen J. Russell, M.D., Ph.D.

President

American Society of Gene & Cell Therapy