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David M. Barrett, J.D.

December 16, 2019

The Honorable Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
2111 Rayburn House Office Building
Washington, DC 20515

Dear Rep. DeGette and Rep. Upton,

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to provide feedback on the Cures 2.0 Call to Action.¹ 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; therefore, the accessibility of such therapies to patients is of paramount importance to ASGCT. The Society supports maximum coverage and reimbursement of approved therapies and payment models that foster patient access. Further discussion will be necessary about the appropriate balance of fair pricing determinations and continued stimulation of innovation, and thus ASGCT does not take positions on any individual pricing decisions.

We acknowledge that there is no one-size-fits-all solution to ensure access and value, which depend on the technology, patient population, and payer mix. Our experience with the few therapies on the market and those in late-stage development has demonstrated that there are several top-line issues that should be addressed in order to facilitate access and encourage innovation. In response to your request for comments regarding how best to reform Medicare coding, coverage, and payment to better support patients' access to innovative therapies,

¹<https://degette.house.gov/sites/degette.house.gov/files/Cures%202.0%20Call%20to%20Action%20Document.pdf>

ASGCT suggests the following areas:

- Eliminating coverage barriers for newly approved therapies
- Reforming the new technology add-on payment system
- Clarifying the pathway to MS-DRGs for new technologies
- Paving the way for alternative payment models

Eliminating Coverage Barriers

As you acknowledge, the FDA has made tremendous progress in working with drug sponsors to make safe and effective treatments available to patients and their families as quickly as possible. This progress, however, has not been uniformly shared among the payer community, which has set up non-medically or scientifically justified barriers to access. This is especially concerning for patients with progressive diseases, for which early administration of a therapy may prevent, but not reverse, morbidities and mortality. Therefore, the potential impact of a product may be diminished if a patient is only able to maintain his/her quality of life at the later time of treatment. Prolonging the negative aspects of current standards of care should also be considered—hospitalizations, infusions, in-home equipment needs, inability to attend work or school, side effects, and poor outcomes. Potential improvements over standards of care on patients' lives should warrant rapid coverage of these products.

For example, earlier this year CMS released a proposed decision memo on the national coverage analysis for chimeric antigen receptor T-cell (CAR T-cell) therapy. While CMS took comments into consideration² and revised the final decision memo to cover CAR-T cell therapies under a national coverage determination (NCD) in accordance with the FDA label, we are concerned that many of the original proposals, if applied to *future* therapies, could limit access:

- Limiting patient eligibility criteria to specific indications, rather than to the FDA label or nationally recognized compendium recommendations. This would require any new indication or new product with a different indication to go through a new coverage process even when used on-label.
- Limiting the site of care beyond the FDA label. This would make access to providers more difficult, especially for rural patients.
- Requiring additional post-marketing requirements beyond the FDA label and a potentially duplicative patient registry. While the Society supports real-world data collection, we believe it should be done in the least burdensome way and in concert with FDA requirements.

Another example is limitations to access specifically in the Medicaid population which have become apparent with newly approved products. Medicaid is the single largest health insurer of U.S. children, especially those with special health needs.² Gene and cell therapies currently on the market and those in development are sometimes the first products ever approved for a rare disease, or will change the treatment paradigm and quality of life for these patients. However, we have seen concerning examples of programs attempting to thwart coverage or place barriers between patients and their families and the treatment their physician is prescribing when that product happens to be high cost. For instance, Massachusetts included a proposal in its 1115

² <https://www.cms.gov/medicare-coverage-database/staticpages/public-comment.aspx?commentID=31729&ReportType=nca>

Medicaid waiver to give the state authority to determine whether to cover a drug approved under the FDA's accelerated approval program. While this proposal was denied, other states have denied coverage of FDA-approved therapies to patients by deeming them experimental for their labeled indication, placing site-of-care coverage restrictions, restricting coverage to clinical trial populations, or de facto denying coverage by requiring prior authorizations that take years. CMS acknowledged this issue and attempted to clarify³ that drugs approved under accelerated approval are FDA-approved drugs and considered covered outpatient drugs for the purpose of Medicaid coverage. While we acknowledge the greater state budgetary issues, it is not appropriate for these programs to deny or delay access to therapy for non-medical reasons, especially given that these therapies are not uniquely expensive for high-value treatments (for example, the average cost of a heart transplant in 2017 was \$1.4 million⁴).

In conclusion, we urge you to consider ways to clarify, educate, and oversee the way CMS and state Medicaid programs are interpreting the law with regard to coverage of FDA-approved therapies and medication management mechanisms to ensure that patients are not being denied access to life-changing and potentially lifesaving products.

CAR T-Cell Therapy and Future Cell Therapies

The approval of CAR-T therapies has tested the readiness of the Medicare program for future innovations in cell and gene therapy. ASGCT believes that we can learn from past problems and improve the CMS process going forward. The current Medicare reimbursement mechanisms for CAR T-cell therapy often leave a significant gap in payment to certified hospitals compared to their combined costs for services and for the biologic therapy. ASGCT is concerned that such losses are unsustainable and disincentivize qualified providers from offering the products. 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. It also discourages future investments in gene therapies if developers do not have a clear path to treating the intended patient population. We suggest the following changes in order to modernize the Medicare program to provide access to these new innovations:

Reassessing the new technology add-on payment

The new technology add-on payment (NTAP) was established in 2001 to facilitate the adoption of new innovations offering clinical improvements. The 50-percent limit in the original formula created nearly two decades ago no longer reflects the costs of new technology and is insufficient to support new healthcare innovations. CMS acknowledged the need to increase the NTAP cap during this year's IPPS rulemaking. While the Society recommended CMS dramatically increase the percentage—to 100 percent—we are appreciative that CMS made some forward progress by setting the new cap at 65 percent. However, we believe that additional progress could be made, especially since CMS has historically not utilized all allocated NTAP funds in previous years. This could be done at little cost to taxpayers.

³ <https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf>

⁴ http://www.milliman.com/insight/2017/2017-U_S_-organ-and-tissue-transplant-cost-estimates-and-discussion/

The new maximum add-on payment of 65 percent is not sufficient to ensure broad adoption of CAR T-cell therapy, with costs that completely exceed the total MS-DRG but that provide extremely high value. The principle of a cap 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.

ASGCT also recommends flexibility in the commencement and duration of the add-on. Current CMS policy allows up to three years of NTAPs after product approval. Depending on the date of product approval by the FDA, there may be a long lag until the NTAP is applied, as applications for the payment are due in October for the upcoming fiscal year. In small patient populations, even the best-case scenario of three years may not be sufficiently long to collect the amount of data needed to accurately inform a new DRG (discussed in greater depth below).

We support a broader conversation on NTAP reform that can facilitate transparency in provider charging practices, greater equity in reimbursement levels to all providers of these therapies, and broader support for the intent of the program for 21st Century medicine. We welcome the opportunity to continue to engage in a dialogue with your offices.

A clear pathway to new MS-DRGs for gene-modified cell therapies

ASGCT foresees the need create new MS-DRGs for gene-modified cell therapies that more accurately and consistently reflect the actual costs to providers. We appreciate that CMS considered new and alternative methods for reimbursing these types of novel products in the 2020 IPPS proposed rule. While future MS-DRGs for gene-modified cell therapies could encompass the costs of the therapeutic products and hospital services provided to the patient (as is currently standard practice), ASGCT recommends that new MS-DRGs reimburse for patient care costs alone, with a separate payment or MS-DRG group for the product. Separating patient care service costs and product costs has several advantages.

1. CMS could continue to use the averaging process that is central to the current prospective payment system to pay for the patient care portion of the total case cost.
2. CMS would have full visibility of product costs.
3. The same “patient care only” MS-DRG could be used across new products.
4. CMS could apply hospital-specific adjustments (which consider geographic location, hospital and patient characteristics, etc.) for the patient care portion, but not for the product which doesn’t carry the same differentials.
5. CMS would have the flexibility to employ value-based product payment models in Medicare Part A in the future.

This approach is similar to approaches taken by the New York State and Massachusetts Medicaid fee-for-service (FFS) programs, which reimburse facilities for a product separately from the bundled payment for services. We recommend that Cures 2.0 gives CMS greater direction in how to structure reimbursement for new gene-modified cell therapies that enables patient access, ensures transparency, and is accountable to taxpayers.

Collection of acquisition cost data

The Society recommends that hospitals be required to report the actual acquisition costs for new therapies, like CAR T, in the new value code 86 field. In the time after a product is approved, it is important that CMS understand how much its providers are spending to procure the product in order to accurately inform future rate setting in a new MS-DRG. Overall charge data does not reveal product discounts, free product given for clinical trial participants, and other variation. Ideally, collection of value code data would be done until rate setting commences, given the often limited populations for these therapies and the need for a robust data set.

Alternative Payment Models

Gene therapy represents a radical shift in our approach to disease treatment. By modifying the expression of a patient's genes or repairing abnormal genes, gene therapy often addresses the root cause of diseases. While several gene and cell therapies have received FDA approvals over the past 20 years, the field has recently experienced a turning point. In 2017, the approvals of three gene therapies for human medical use in the U.S. for a rare inherited retinal disorder and certain types and indications of blood cancers marked a turning point in the field, with subsequent approvals and many more likely on the way. Many of the currently approved gene therapies and those in the pipeline are anticipated to involve a single administration of treatment. This represents a new paradigm for payers, as our current drug payment system has evolved around treatments that are administered over the course of a patient's disease—which can be a lifetime—to mitigate symptoms.

Many payment models have been proposed to enable patient access while addressing payer ability to cover upfront costs, and we are supportive of these efforts. These include:

- Linking payment to treatment outcomes—with lower costs being incurred for less effective individual patient results—as a voluntary option for manufacturers. This reduces or eliminates a payer's exposure to the cost of the product if a patient does not respond to treatment with a certain clinical outcome, so that lower costs are incurred for less effective individual patient results.
- Offering payment for treatments over a longer duration of time.

While value-based and longer-term payment arrangements are being tested in limited markets, broad adoption and greater risk sharing is currently limited by the Medicaid Best Price program, which requires drug manufacturers to give Medicaid the best price given to any other purchaser (by providing it with a mandatory rebate of 23.1 percent of the average manufacturers' price or, if another purchaser is offered a greater rebate, that greater rebate amount). Value-based payment agreements and long-term financing models may be prevented by Medicaid Best Price requirements because, if a manufacturer offers an outcomes-based price with a deeper discount than 23.1 percent, or an installment plan with payments that are a fraction of the total cost of the product per payment, the manufacturer would be required to offer that same low price to all Medicaid programs for all Medicaid beneficiaries, regardless of whether they are part of one of these novel arrangements or what the individual outcomes are. ASGCT supports exploration of modifications and/or clarifications to Medicaid Best Price requirements that could facilitate the development of more value-based payment and/or installment arrangements.

Linking drug payment to outcomes incentivizes innovation and patient follow up. There are creative models some manufacturers would like to pursue to tie a portion of the cost of the therapy to successful and/or durable outcomes. There are yet others that would like to incentivize use of their product by offering to pay for current patient standard of care if the new therapy does not work or fails after a period. These scenarios, while a positive step for patients and payers, are hindered by the current drafting of the Stark and Anti-Kickback laws which can view these types of arrangements as illegal kickbacks. ASGCT strongly supports guardrails to prevent corruption in the medical system but believes that updating these laws is critical to enabling the type of risk sharing and patient engagement crucial to advancing novel therapies.

Conclusion

ASGCT appreciates your thoughtful consideration of how to modernize coverage and access for new innovative therapies, including gene and cell therapies. ASGCT believes that the following options, combined, offer a better path forward for patients and their families and will help continue to stimulate innovation in this exciting scientific field:

- Eliminating coverage barriers for newly approved therapies
- Reforming the new technology add-on payment system
- Clarifying the pathway to MS-DRGs for new technologies
- Paving the way for alternative payment models

Please contact Betsy Foss-Campbell, Director of Policy and Advocacy, with any questions at bfoss@asgct.org. We look forward to engaging with you in your legislative development process.

Sincerely,



Guangping Gao, PhD
President