



Seema Verma  
Administrator  
Centers for Medicare & Medicaid Services  
U.S. Department of Health and Human Services  
Hubert H. Humphrey Building  
200 Independence Ave, SW  
Washington, DC 20201

**RE: Medicare Program; Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2020 Rates; Proposed Quality Reporting Requirements for Specific Providers; Medicare and Medicaid Promoting Interoperability Programs Proposed Requirements for Eligible Hospitals and Critical Access Hospitals.**

The Alliance for Regenerative Medicine (ARM) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS) Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long Term Care Hospital Prospective Payment System and Proposed Policy Changes and Fiscal Year 2020 Rates Proposed Rule (Proposed Rule).<sup>1</sup> Specifically, we are writing to thank CMS for all of its proposals and thoughts related to modifying Medicare's New Technology Add-on Payment (NTAP) Program. ARM appreciates that CMS has discussed a few options to improve the current NTAP and overall MS-DRG system with a focus on creating a methodology and a system that balances appropriate access and cost effective care to new and innovative therapies. ARM looks forward to working with CMS to create a transparent and predictable NTAP and MS-DRG system that will continue to stimulate and reward innovation in the inpatient setting.

ARM is an international multi-stakeholder advocacy organization that promotes legislative, regulatory, and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine worldwide. ARM comprises more than 300 leading life sciences companies, research institutions, investors, and patient groups that represent the regenerative medicine and advanced therapies community. ARM takes the lead on the sector's most pressing and significant issues, fostering research, development, investment, and commercialization of transformational treatments and cures for patients worldwide. The regenerative medicine and advanced therapies sector is the next frontier in the fight against some of humankind's most devastating diseases and disorders. As of year-end 2018, ARM estimates there are 906 regenerative medicine and advanced therapies developers worldwide sponsoring 1,028 clinical trials across dozens of

---

<sup>1</sup> 84 Fed. Reg. 19158 (May 3, 2019).

indications, including oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.<sup>2</sup>

A subset of these clinical trials focuses on the power of chimeric antigen receptor (CAR T) therapies. These therapies are the first in a wave of new and exciting advanced therapies and technologies that are the next frontier in the fight against some of humankind's most devastating diseases and disorders. CAR T therapy is a type of treatment in which a patient's T cells (a type of immune system cell) are changed in the laboratory so they will attack cancer cells. T cells are taken from a patient's blood, as it flows through a tube to an apheresis machine, which removes the white blood cells, including the T cells, and sends the rest of the blood back to the patient. Then, the gene for a special receptor called a chimeric antigen receptor (CAR) is inserted into the T cells in the laboratory. Millions of the CAR T cells are grown in the laboratory and then given to the patient by infusion. The CAR T cells are able to bind to an antigen on the cancer cells and kill them.<sup>3</sup> ARM is currently tracking the outcomes of the approximately 158 ongoing clinical trials using the CAR T technology in a variety of stages of cancer and cancer types. ARM believes that this new and promising technology provides the possibility that most future treatments for many types of cancer at its many stages will focus on using the power of the patient's immune system to fight their particular disease.

What is critical about all the technologies represented by ARM, including CAR T, immunotherapy, and cell and gene therapy, is that many of the products are transformative – they provide a durable therapeutic benefit or even a cure with a single administration of the therapy. The potential for dramatic clinical benefit is why these innovations are changing medical care and must be considered as part of the solution and not as part of the problem of rising overall drug costs. ARM believes that we are at the beginning of our scientific journey to curing many of these diseases and urges CMS to work with all stakeholders in order to streamline and ensure broad and safe beneficiary access to these classes of therapies as well as other cutting edge treatments in the inpatient setting.

## **I. Executive Summary:**

- CMS should adjust its CAR T specific reimbursement policies to utilize actual drug acquisition costs, rather than marked up charges in NTAP and outlier payment calculations for fiscal year (FY) 2020. This will avoid a hospital's need to significantly mark up the costs associated with acquiring the CAR T therapy and encourage broader patient access to CAR T therapy.
- CMS should also finalize policies for FY 2020 that create the infrastructure to move toward an appropriately valued CAR T MS-DRG in future fiscal years, including the generation of accurate data required for future rate setting.

---

<sup>2</sup> <https://alliancerm.org/publication/2018-annual-report/>

<sup>3</sup> <https://www.cancer.gov/publications/dictionaries/cancer-terms/def/CAR-T-cell-therapy>

- CMS should recognize certain FDA approval designations for drugs as dispositive for newness and substantial clinical improvement.
- ARM disagrees with CMS' proposal to change the severity level designation for 13 ICD-10-CM diagnosis codes from categories I21 and I22 from a MCC to a CC.
- CMS should establish a more frequent NTAP process.

## **II. ARM Agrees with CMS that the Agency Should Not Yet Establish a New MS-DRG for CAR-T Therapies**

CMS proposes not to modify the current MS-DRG assignment for cases reporting CAR T-cell therapies for FY 2020.<sup>4</sup> ARM agrees with the Agency's reasoning, which focuses on lack of data as stated by CMS that "we do not have the comprehensive clinical and cost data that we generally believe are needed to do so."<sup>5</sup> The Agency adds that "we expect that, in future years, we would have additional data that exhibit more stability and greater consistency in charging and billing practices that could be used to evaluate the potential creation of a new MS-DRG specifically for cases involving CAR T-cell therapies."<sup>6</sup> ARM agrees with this conclusion but only to the extent that CMS establishes, as detailed below, reimbursement policies for FY 2020 that generate robust and accurate clinical and cost data for future rate setting.

For FY 2021 and beyond, ARM believes that the creation of a new MS-DRG would establish a transparent and predictable reimbursement infrastructure for providers that would mitigate or avoid significant financial losses. The new MS-DRG would be a stable approach towards reimbursing new CAR T therapies that will help promote access to these therapies in the inpatient setting. ARM, however, urges CMS to include both the therapy costs and all of the associated care services for the delivery of the CAR T within a comprehensive reimbursement approach. Without these important and associated costs, the new MS-DRG and overall approach would not achieve its intended purpose of providing appropriate reimbursement and subsequent patient access to these novel treatments. Finally, ARM notes that many Medicaid programs and commercial insurers rely on CMS' policies to establish reimbursement rates for Medicaid and commercial patients such that a new MS-DRG would also have a positive impact on access within these insurance programs.

## **III. CAR T is a Unique Technology Requiring Unique Reimbursement Solutions to Ensure Access**

CAR T technology is at an early stage. However, even at this early stage, it is clear that CAR T-cells have the potential to dramatically improve patient outcomes. CAR T therapies are highly specific and differentiated. They are

---

<sup>4</sup> 84 Fed. Reg. 19181.

<sup>5</sup> Id.

<sup>6</sup> Id.

personalized for an individual patient and the CAR T technologies are significantly different from one another. Among other things, the CAR design, vector used for genetic transfer, and manufacturing process can all vary substantially between therapies because each CAR T therapy must be tailored to treat a unique combination of clinical indications, safety profiles, and patient populations in order to provide a therapy that is both effective and personalized for each unique patient.

It is critical that Medicare's reimbursement policy for CAR T recognizes the significant power and uniqueness that this technology brings to patient care. This deep level and type of personalized medicine is the future of patient care. ARM believes that just like treatments for devastating diseases are evolving so should CMS' payment policies for the treatments for these diseases. For example, the current IPPS system fundamentally relies on reducing charges to cost based on an individual hospital's cost-to-charge ratio (CCR) plus other hospital specific characteristics, such as geographic location, percentage of uninsured, and teaching/non-teaching hospital to establish a particular reimbursement amount in addition to the DRG payment for each discharge. ARM believes that CMS' reimbursement policies for CAR T, and cell and gene therapies more broadly, should focus on a hospital's acquisition cost, via a uniform maximum methodology, within CMS' payment formulas. This new approach will create a more level playing field to ensure access for all Medicare beneficiaries as more hospitals will be able to provide these therapies. This new reimbursement formula, based on acquisition cost and not the traditional CCR is a more transparent, predictable, and is a fairer approach, as detailed below for all providers.

#### **IV. For Fiscal Year 2020 CMS Should Prioritize Appropriate Reimbursement for CAR T Therapies While Also Ensuring the Collection of Accurate Data for Future Rate Setting**

As a general principle, ARM believes that CMS' final payment methodology must be practicable from an implementation point of view and consistent with the current coding requirements of all providers. ARM appreciates CMS' various statements on how to appropriately reimburse providers for administering CAR T therapies. As illustrated above, administering a CAR T therapy is a capital and labor intensive process requiring many types of services, expertise, and resources. To effectuate the Agency's goal of appropriate reimbursement, ARM believes that CMS must implement a solution that focuses on drug acquisition cost in the calculation of both the NTAP and outlier payment. In doing so, CMS will dramatically reduce the current wide variation of submitted costs due to differences in charging practices by providers for CAR T therapies in order to receive adequate reimbursement.<sup>7</sup> In addition to all of the costs associated with administering a CAR T, ARM notes that during FY 2020 and beyond providers will likely be implementing CMS' Coverage with Evidence Development (CED) for CAR T therapies. The CED will create additional administrative costs for each CAR T administration further emphasizing

---

<sup>7</sup> Id.

the importance of establishing an appropriate reimbursement rate for FY 2020 and beyond.

*A. ARM Urges CMS to Make a Uniform Add-On Payment That Equals 80 Percent of the Cost of the CAR T Therapy*

ARM appreciates the Agency's proposals and thoughts around establishing a unique reimbursement formula for CAR T therapies. In finalizing this payment amount, ARM believes that CMS' final payment policy must comply with Congressional intent when it created the NTAP to establish an additional payment that adequately reflects the estimated average cost of such service or technology.<sup>8</sup> Further, Congress instructed CMS that this additional payment might be satisfied by means of a new technology group known as an "add-on payment," that is, a payment adjustment or any other similar mechanism for increasing the amount as long as it represents the estimated average cost of such service or technology.<sup>9</sup> The statute provides clear instruction to CMS to reimburse providers an amount that represents as estimated average cost of the technology. As stated above, ARM urges CMS to transition from the current CCR methodology, which is not focused on the estimated acquisition cost to focus on paying hospitals consistent with the statutory requirement.

As such, ARM urges CMS to finalize its proposal to reimburse providers of CAR T based on a uniform add-on payment policy. ARM believes that this should be the primary focus of the payment changes for CAR T administrations given the unique nature of this therapy and the significant clinical promise this therapy offers beneficiaries. CMS proposes that this uniform rate be "65 percent of the cost of the technology."<sup>10</sup> CMS seeks public comment on this alternative approach for CAR T therapies and ARM believes that this, methodological approach satisfies many of the Agency's and stakeholder objectives of reimbursing based on acquisition cost, satisfying Congressional intent, and providing providers with a transparent and predictable reimbursement amount for each CAR T administered.

ARM, however, urges CMS to increase this rate to 80 percent of the cost of the technology. Based on ARM's data analysis, 65 percent would still require many hospitals to significantly mark-up the cost of the CAR T in order to break even; whereas, with a uniform maximum at 80 percent those hospitals with more conservative marking-up practices can still provide access to beneficiaries. ARM, therefore urges CMS to finalize a payment methodology for CAR T therapies that focuses on a uniform maximum payment amount that reduces the need for all hospitals to mark-up the cost of the CAR T. This approach reduces incentives for hospitals to significantly mark-up the therapy to recoup their costs because it neutralizes the "lessor of" provision in the current NTAP formula and will hopefully allow for broader access for all Medicare beneficiaries.

---

<sup>8</sup> SSA §1886(d)(5)(k)(ii)(III).

<sup>9</sup> SSA §1886(d)(5)(k)(v).

<sup>10</sup> 84 Fed. Reg. 19182.

Recently, new revenue and value codes were established by the National Uniform Billing Committee (NUBC) that ARM believes could dramatically improve the collection of cost data.<sup>11</sup> ARM encourages CMS to utilize these new codes in its final payment methodology to ensure more appropriate payment for all NTAP cases. Specifically, with the implementation of new revenue code 0891<sup>12</sup> CMS will have the charge information for cell therapy products (at present one of two CAR-T products) and with value code 86, which CMS should require of all hospitals, CMS will be able to capture actual acquisition cost information for cell and gene therapies. With this information, CMS should have the necessary information to appropriately reimburse hospitals for FY 2020 and establish an accurately paying MS-DRG for CAR T therapies for FY 2021 and beyond.

*B. CMS Should Also Focus on Acquisition Cost In Calculating A Potential Outlier Payment For CAR T Cases*

CMS solicits comments on the use of a CCR of 1.0 to calculate outlier payments. Specifically, the Agency states that “in light of the additional experience with billing and payment for cases involving CAR T-cell therapies to Medicare patients, we should consider utilizing a specific CCR for ICD-10-PCS procedure codes used to report the performance of procedures involving the use of CAR T-cell therapies; for example, a CCR of 1.0, when determining outlier payments, when determining the new technology add-on payments, and when determining payments to IPPS-excluded cancer hospitals for CAR T-cell therapies.”<sup>13</sup> ARM supports CMS’ efforts to accurately reimburse hospitals for their cost of care while also balancing access to innovation and managing overall drug costs. For these reasons and those stated above, ARM urges CMS to also use drug acquisition cost to calculate the outlier payment, consistent with the policy approach of a CCR of 1.0.

Specifically, ARM recommends that CMS subtract the drug charges in revenue code 0891 from the total charges on the claim and then apply the hospital operating CCR to the remainder in order to estimate the patient care costs of the case. CMS would then add the CAR T therapy acquisition cost as identified either with the value code or by using ASP, for those hospitals that did not comply with the proposed requirement, to obtain a total cost of the claim. Then, for purposes of calculating the outlier payment, CMS would use this sum (of total cost) and compare it to the sum of the MS-DRG payment, the NTAP, and the outlier threshold and reimburse any difference at 80 percent without a geographical adjustment.

ARM believes that this new process, the combination of a uniform maximum NTAP of 80 percent as well as the revamped outlier calculation described above are a logical outgrowth of a CCR of 1.0 which simply allows CMS to neutralize the issues of mark-up and pay outlier appropriately irrespective of variable hospital mark-up practices of the product. These policies taken together will accomplish many

---

<sup>11</sup> <http://www.nubc.org/subscribersonly/PDFs/Cell%20Therapy%20Changes%20August%202018.pdf>

<sup>12</sup> Special Processed Drugs-FDA Approved Cell Therapy-Charges for Modified cell therapy.

<sup>13</sup> 84 Fed. Reg. 19182.

important objectives. First, it will promote greater price transparency, which is a goal of the Administration. Second, it will comply with the statutory intent of the NTAP to reimburse based on average acquisition cost. Third, the policy will avoid overpayment of outliers as a result of inconsistent charging practices. Fourth, this will allow for the collection the necessary cost data to establish a new and accurately paying CAR T MS-DRG in the future. Finally, the policy should satisfy CMS' goal of appropriately incentivizing the utilization of new technologies by all eligible providers.

## **V. CMS Should Establish a New MS-DRG For CAR-T Therapies in FY 2021**

Congress also provided CMS the authority to create "a new technology group" to effectuate additional payment for new technologies eligible for a NTAP.<sup>14</sup> CMS states that in considering a new MS-DRG, "we consider whether the resource consumption and clinical characteristics of the patients with a given set of conditions are significantly different than the remaining patients in the MS-DRG."<sup>15</sup> CMS further adds that "in evaluating resources costs, we consider both the absolute and percentage differences in average costs between the cases we select and review the remainder of cases in the MS-DRG."<sup>16</sup> ARM believes that given the resources used for MS-DRG 016, the impact of CMS' rate-setting methodology, namely charge compression, and the clinical characteristics of the patients assigned to MS-DRG 016 as compared to CAR-T therapies warrants that not only should CMS establish a new MS-DRG for these patients, but that it should do it in a different manner. The clinical characteristics, treatment process, side effects and resource utilization for patients with diffuse large B-cell lymphoma (DLBCL) who receive CAR T differs significantly from those patients receiving a bone marrow transplant as characterized by MS-DRG 016. Specifically, patients receiving CAR T cells may have worse comorbidities and also have fewer treatment options for their disease than patients receiving autologous stem cell transplants (ASCT). For example, high-dose chemotherapy with ASCT is used as a second-line treatment for patients who have responded to first-line therapy and then have experienced a relapse. Patients eligible for ASCT have chemosensitive disease and are generally younger and have fewer comorbidities than patients who are not eligible for ASCT. As a result of these clinical differences, there is a significantly greater resource utilization for CAR T cases relative to other cases in MS-DRG 016. Therefore, ARM urges and agrees with CMS to focus on creating a new MS-DRG for CAR T cases for FY 2021.

As stated above, an accurate and appropriately reimbursed MS-DRG results from accurate cost and clinical data. Therefore, as an initial matter, ARM urges CMS to not include clinical trial cases in any future relative weight calculation. ARM shares the Agency's concerns regarding the data because "the absence of the drug costs on claims for cases involving clinical trial claims could have a significant

---

<sup>14</sup> SSA §1886(d)(5)(k)(v).

<sup>15</sup> 84 Fed. Reg. 19172.

<sup>16</sup> *Id.*

impact on the relative weight.”<sup>17</sup> Further, many clinical trial costs could be paid for by the trial sponsor and therefore not appear on the claim, which additionally complicates using clinical trial cases for rate setting purposes.

Consistent with ARM’s policy suggestions for uniform NTAP payment and a new outlier payment methodology, ARM urges CMS to use acquisition cost for future rate setting purposes. ARM hopes that for FY 2020, CMS will require hospitals to submit their acquisition cost data via value code 86 so that CMS has better cost information to use in computing a future relative weight for a new CAR T MS-DRG.

CMS also seeks comment on whether it would be appropriate to alter how the IPPS payment adjustments are determined for the wage index, indirect graduate medical education costs, and the costs of treating a high percentage of uninsured patients. Several examples are included in the proposed rule where applying these adjustments to a new MS-DRG with payments of \$400,000 would yield exceptionally high adjustment payments to the hospital. CMS specifically cites its exceptions authority under section 1886(d)(5)(I) as a possible legal basis for such an adjustment.

As noted above in our comments, there are unique challenges with paying for CAR T therapies under the averaging principles of IPPS. The approach described above for capturing the therapy acquisition costs could enable accurate payments through the establishment of a new MS-DRG, and ARM encourages CMS to undertake such further exceptions to the standard IPPS formula as may be needed to allow hospitals to make these lifesaving therapies available to their patients without the need to dramatically increase its charges.

The creation of a new MS-DRG would establish a transparent and predictable reimbursement infrastructure for providers that would mitigate or avoid significant financial losses. The new MS-DRG would be a stable approach towards reimbursing new CAR T therapies that will help promote access to these therapies in the inpatient setting.

## **VI. ARM Supports CMS’ Effort to Improve The NTAP Program to Incentivize the Use of New Technologies**

In 1983 when Congress created the Inpatient Prospective Payment System, regenerative and advanced technologies were closer to science fiction than the clinical reality they are today. As such, Congress likely did not find the need to include a mechanism or methodology that adequately reimburses hospitals for providing these types of new and innovative technologies. However, in efforts to recognize the value of new technologies, Congress, in 2000, required CMS to establish a mechanism to recognize the costs of new medical services and

---

<sup>17</sup> 84 Fed. Reg. 19181.



technologies in the inpatient setting for discharges beginning on or after October 1, 2001.<sup>18</sup>

Specifically, Congress instructed CMS to “provide for additional payment...in an amount that adequately reflects the estimated average cost of such service or technology.”<sup>19</sup> Further, Congress instructed CMS that this additional payment might be satisfied by means of a new technology group known as an “add-on payment,” that is, a payment adjustment or any other similar mechanism for increasing the amount as long as it represents the estimated average cost of such service or technology.<sup>20</sup>

Congress also required that the new technology represent an advance in medical technology that substantially improves the diagnosis or treatment of individuals. As stated above, regenerative medicine and advanced therapies on the market and in the pipeline epitomize Congress’ statement on new technologies. Regenerative, cell, gene and immune-therapies have already and will continue to demonstrate substantial clinical improvement by improving health outcomes and hold the promise of reducing overall health care costs. Hundreds of next generation medicine products in clinical trials hold similar promise to treat unmet medical needs, improve patient care, and bend the health care cost curve in ways that current forms of clinical care have not been able to achieve. Many of the diseases targeted by researchers and product developers, such as heart disease, diabetes and musculoskeletal conditions, are chronic conditions that affect millions of American families and are significant cost drivers for Medicare.

In enacting the NTAP program Congress surely did not intend the NTAP program to be a barrier rather than a facilitator of access to new therapies and technologies. Therefore, ARM appreciates CMS’ efforts to update some of the NTAP’s eligibility criteria and change the current reimbursement rate to be more in line with Congressional intent. ARM believes that without improving the NTAP program, many of the technologies described above will be out of reach for Medicare beneficiaries, or worse, never be developed due to CMS’ insufficient eligibility criteria and payment rate.

*A. ARM Supports CMS’ Effort to Increase the Payment Rate for Therapies that Receive a NTAP, but 65 Percent is Not Enough*

ARM thanks CMS for its transparency, ideas, and efforts to change its current reimbursement rate for the overall NTAP program. ARM agrees with the Agency’s statement “that we agree that there may be merit to the recommendations to increase the maximum add-on amount and that capping the add-on payment amount at 50 percent could, in some cases, no longer provide a sufficient incentive for use of the new technology.”<sup>21</sup> ARM appreciates that when CMS developed the 50

---

<sup>18</sup> SSA §§ 1886(d)(5)(K) and (L).

<sup>19</sup> SSA §1886(d)(K)(ii)(III).

<sup>20</sup> SSA §1886(d)(K)(v).

<sup>21</sup> 84 Fed. Reg. 19162.

percent standard, it could not have envisioned the power of the next generation of therapies. It is that unprecedented clinical benefit, however, that provides the rationale for why CMS must change its payment approach for these therapies to increase the payment rate as the current methodology is not an accurate valuation of these new technologies nor does ARM believe consistent with Congressional intent. Rather, the current rate provides a dis-incentive for these products to be used in the hospital setting. That, in turn, limits patient access to these products in the short term and could stifle the development of similar therapies in the long term.

In response to this concern, CMS proposes “to modify the current payment mechanism to increase the amount of the maximum add-on payment amount to 65 percent.”<sup>22</sup> Specifically, the Agency proposed that, beginning with discharges occurring on or after October 1, 2019, if the costs of a discharge involving a new medical service or technology exceed the full DRG payment (including payments for IME and DSH, but excluding outlier payments), Medicare would make an add-on payment equal to the lesser of: (1) 65 percent of the costs of the new medical service or technology; or (2) 65 percent of the amount by which the costs of the case exceed the standard DRG payment.<sup>23</sup> ARM thanks CMS for proposing this increase but believes that this increase is insufficient.

The overall NTAP reimbursement formula deflates the overall amount because it focuses on an amount that is the “lesser of” two calculations. As stated above, Congress instructed CMS to reimburse hospitals an amount that reflects the estimated average cost of the technology. ARM respectfully disagrees that the new proposed payment rate within the current “lessor of” formula satisfies Congressional intent. Merely increasing the rate to 65 percent within the current formula does not equal the statutory requirement of reimbursing based on the average cost of the technology.

Further, ARM believes that this new rate would not even meet the Agency’s objectives, which is to provide a sufficient incentive to use the new technology given the way the formula is operationalized. The additional fifteen percent increase is not enough to sufficiently cover hospital costs to the point that the hospital can even manage the costs of the technology within the larger prospective payment system. As such, ARM believes that the proposed increase would not incentivize the adoption of new technologies. ARM, therefore, suggests that CMS increase the rate to at least 80 percent as this comes closer to meeting Congressional intent, is consistent with current outlier payment policy, and the Agency’s proposed access goals.

## **VII. Similar to Devices, CMS Should Recognize Certain FDA Approval Designations For Drugs As Dispositive for Newness and Substantial Clinical Improvement NTAP Criteria**

---

<sup>22</sup> *Id.*

<sup>23</sup> *Id.*

CMS states that the “Administration is committed to addressing barriers to healthcare innovation and ensuring Medicare beneficiaries have access to critical and life-saving new cures and technologies that improve beneficiary health outcomes.”<sup>24</sup> ARM fully supports this initiative and believes that some of the inpatient setting’s current NTAP policies, in addition to payment, hamper access and should also be the focus of the Administration’s goals to promote access.

To demonstrate this commitment, CMS proposes a dramatic change in the eligibility criteria for certain devices but not for drugs or biologicals that meet a very similar evidentiary standard. Specifically, CMS proposes that starting in fiscal year 2021, if a medical device is part of the FDA’s Breakthrough Devices Program and received FDA marketing authorization, it would be considered new and not substantially similar to an existing technology for purposes of the new technology add-on payment under the IPPS.<sup>25</sup> Additionally, CMS states that because the technology may not have a sufficient evidence base to demonstrate substantial clinical improvement at the time of FDA marketing authorization, the medical device would not need to meet the substantial clinical improvement requirement.<sup>26</sup> ARM urges CMS to finalize this policy and also urges the Agency to add drugs and biologicals (drugs) to its policy change. Such an approach would signal support for more and better patient access to transformative medical devices and drugs consistent with the intent of the *21<sup>st</sup> Century Cures Act*.

In support of its conclusion to exclude drugs from this significant policy proposal, CMS states that “current drug-pricing system provides generous incentives for innovation, but too often fails to deliver important medications at an affordable cost.” Making this policy applicable to drugs would further incentive innovation but without decreasing cost, a key priority of this Administration.<sup>27</sup> ARM respectfully disagrees. These broad and sweeping statements regarding incentives for innovation are inconsistent with the specific statements and subsequent Agency policy proposals related to NTAP payment. CMS is proposing to change the NTAP because it knows that the current payment system does not promote access to new and innovative technologies that have already demonstrated significant clinical impact. ARM urges CMS to be consistent in its approach to promoting access to all innovative technologies.

Congress required that the new technology represent an advance in medical technology that substantially improves the diagnosis or treatment of individuals.<sup>28</sup> As stated above, regenerative medicine and advanced therapies on the market and in the pipeline epitomize Congress’ statement on new technologies. Regenerative therapies, such as CAR T, have already and will continue to demonstrate substantial clinical improvement by improving health outcomes and hold the promise of reducing overall health care costs. Hundreds of regenerative medicine products in

---

<sup>24</sup> 84 Fed. Reg. 19372.

<sup>25</sup> *Id.*

<sup>26</sup> *Id.*

<sup>27</sup> 84 Fed. Reg. 19672.

<sup>28</sup> 83 Fed. Reg. 20279.

clinical trials hold similar promise to treat unmet medical needs, improve patient care, and bend the health care cost curve in ways that current forms of clinical care have not been able to achieve. Many of the diseases targeted by regenerative medicine researchers and product developers, such as heart disease, diabetes, and musculoskeletal conditions, are chronic conditions that affect millions of American families and are major cost drivers for Medicare.

Congress, however, did not require the new medical technology to be a novel mechanism of action, to treat a different patient population, or have a certain clinical trial size. These criteria were developed by CMS, and ARM believes that CMS should update and/or eliminate many of these criteria, just as it is proposing to do for certain devices.

**A. Breakthrough Therapy or Regenerative Medicine Advanced Therapy (RMAT) Designation Should be Dispositive for the Newness and Substantial Clinical Improvement NTAP Criteria for Drugs or Biologicals**

CMS notes that the Agency “evaluates whether the use of the device, drug, service or technology significantly improves clinical outcomes for a patient population as compared to currently available treatments”<sup>29</sup> as a determining factor of substantial clinical improvement. ARM previously stated and continues to believe that this standard was created by Congress and CMS for medical devices as that was the prevailing new technology of the time. This standard, however, should not be applied to regenerative medicine therapies because these criteria are likely outside Congressional intent because it is inconsistent with some of the congressionally created FDA approval rules related to expedited approval programs. Specifically, the FDA defines the congressionally created “breakthrough therapy” and designates a therapy as such if it “may demonstrate substantial improvement over existing therapies.” In addition, the Regenerative Medicine Advanced Therapy (RMAT) designation is granted to products that are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and if clinical evidence shows that it has the potential to meet an unmet medical need. ARM, therefore, believes that CMS’ substantial clinical improvement criteria should not apply to any therapy that has a Breakthrough or RMAT designation from the FDA.

In a previous response to ARM’s request, CMS stated that “if the technology has a status designated by the FDA that is similar to the standards and conditions required to demonstrate substantial clinical improvement under the new technology add-on payment criterion, or is designated as a breakthrough therapy, the technology should be able to demonstrate with evidence that it meets the new technology add-on payment substantial clinical improvement criterion.”<sup>30</sup> ARM appreciates CMS’ stated connection between the FDA designation and its belief that the technology “should be able to demonstrate substantial clinical improvement criterion.” ARM, however, questions why CMS continues to raise concerns regarding

---

<sup>29</sup> 84 Fed. Reg. 19368.

<sup>30</sup> 83 Fed. Reg. 20279.

the substantial clinical improvement criterion for each application that has a Breakthrough or RMAT designation from the FDA and also fails to make this connection for medical devices.

In raising concerns with each NTAP application that has one of the aforementioned FDA designations, it seems to ARM that CMS questions the validity of the FDA designation and the ability of the technology to meet the substantial clinical improvement criterion, which was just satisfied via FDA designation. For example, CMS continues to raise patient mortality data and few published results showing survival benefit as concerns for satisfying substantial clinical improvement. Yet, the FDA designated the therapy as RMAT or Breakthrough because it demonstrated substantial clinical improvement based on these same characteristics and then approved it based on the same criteria. The FDA has the authority to revoke the designation should the Agency believe that the therapy no longer meets this criterion such that if the NTAP applicant was approved with a FDA Breakthrough or RMAT designation it should be definition satisfy the substantial clinical improvement criterion.

In recent NTAP applications, CMS has questioned how clinical improvement can be measured and achieved via the small clinical trials that generated FDA approval. ARM is concerned that this view sets a dangerous precedent by significantly undervaluing new transformative therapies. Cell and gene therapies often target small patient populations as developers are attempting to cure rare diseases or previously untreatable subsets of patients. Therefore, by necessity, the sizes of clinical trials for these products will be small and frequently can include surrogate measures of efficacy, with long-term post-approval patient follow-up expected. The FDA recognizes this and often only requires single-arm trials with small numbers of patients for these products. It is often not feasible for product developers to provide data on a large number of patients, especially those working in rare diseases, as many regenerative and advanced therapeutic developers are. Given the transformative nature of the products, this should not be a reason for CMS to ever deny an NTAP payment.

Similar to the substantial clinical improvement requirement, ARM believes that the current newness criteria are inappropriate for regenerative and advanced therapies. Specifically, CMS established the additional criteria requiring an applicant to show its technology is not “substantially similar” to existing technologies and does not treat the same or similar disease. As noted earlier, products that receive Breakthrough or RMAT designations are by definition determined by the FDA to be an improvement over existing therapies or treat unmet medical needs. If FDA makes this determination, it would be inconsistent for CMS to make a clinical determination that such a product is “substantially similar” to an existing product. Moreover, given the incremental nature of technological advancement, the ability of CMS to determine when a product meets a “newness” standard is not clear.

ARM believes that by continuously raising patient mortality data and few published results showing survival benefit as concerns for the NTAP, CMS seems to be contradicting itself. First, the Agency states that the same data that the FDA

relied upon for Breakthrough or RMAT designation and subsequent FDA approval should suffice for the NTAP. Yet, the Agency then questions patient mortality data and few published results showing survival benefit as concerns related to eligibility for the NTAP. ARM believes that CMS cannot state that the technology should have no problem meeting substantial clinical improvement standard for NTAP approval while simultaneously questioning the same data used to demonstrate FDA designation and approval. To reconcile this contradiction, ARM believes that if the FDA approved a therapy with a Breakthrough or RMAT designation and has not revoked the designation, the substantial clinical improvement criterion should automatically be satisfied.

### **B. Clinical Trial Size on a FDA Approved Therapy Should Never Disqualify a NTAP Application**

In addition, in recent NTAP applications, CMS has questioned how clinical improvement can be measured and achieved via the small clinical trials that generated FDA approval. ARM is concerned that this view sets a dangerous precedent by significantly undervaluing new transformative therapies. Cell and gene therapies often target small patient populations as developers are attempting to cure rare diseases or previously untreatable subsets of patients. Therefore, by necessity, the sizes of clinical trials for these products will be small and frequently can include surrogate measures of efficacy, with long-term post-approval patient follow-up expected. The FDA recognizes this and often only requires single-arm trials with small numbers of patients for these products. It is often not feasible for product developers to provide data on a large number of patients, especially those working in rare diseases, as many regenerative and advanced therapeutic developers are.

In response, CMS states that “it accepts different types of data (for example, peer-reviewed articles, study results, or letters from major associations, among others) that demonstrate and support the substantial clinical improvement associated with the new medical service or technology’s use. In addition to clinical data, we will consider any evidence that would support the conclusion of a substantial clinical improvement associated with a new medical service or technology.”<sup>31</sup> ARM appreciates that the Agency considers a wide range of data to support substantial clinical improvement but given the FDA approval process and the nature of clinical trial design for this class of transformative products, small clinical trial size should never be a reason for CMS to deny an NTAP.

Consistent with medical devices with Breakthrough designation, ARM believes that the substantial clinical improvement standard is an inappropriate clinical standard for the family of regenerative therapies. Each technology meets very similar evidentiary standards at the FDA and should therefore be treated the same by CMS.

---

<sup>31</sup> *Id.*

## **VIII. Diseases of the Circulatory System Chapter Codes**

In the Diseases of the Circulatory System chapter of the ICD-10-CM diagnosis classification (I00-I99), CMS proposes to change the severity level designation for 13 ICD-10-CM diagnosis codes from categories I21 (Acute myocardial infarction) and I22 (Subsequent ST elevation (STEMI) and non-ST elevation (NSTEMI) myocardial infarction) from an MCC to a CC.

ARM disagrees with the Agency's proposal because ARM believes that these codes significantly impact resource use. In CMS' analysis of MI as primary or secondary, CMS states that CMS will use codes that indicate STEMI or NSTEMI<sup>32</sup>. However, ICD-10-CM codes I21.4 or I22.4 (NSTEMI) are not included in the analysis even though these codes are currently on the 2019 MCC list.<sup>33</sup> If applied, this would mean that STEMIs would be considered CC when in the secondary position and NSTEMIs MCC. This does not align with clinical experience. Including the omitted ICD-10-CM codes I21.4 and I22.4 in the analysis, ARM found 198,204 claims with secondary diagnoses of I21.x or I22.x out of a total of 468,781 claims in calendar year 2016 using the Inpatient Standard Analytic Files.

Additionally, patients are at high risk for death, require on-going monitoring, may need cardiac support, coronary intervention (s), etc. For example, a patient with a pulmonary embolism and a STEMI could require significant increase in the amount of resources compared to a patient with a pulmonary embolism alone. Therefore, ARM strongly urges CMS to delay any action until at least the 2021 Proposed Rule, allowing for more time to assess all data and further consider the magnitude of the implications that this proposed change will have on hospitals and Medicare patients.

## **IX. CMS Should Establish a More Frequent NTAP Process**

Earlier this year Administrator Verma announced, "a comprehensive strategy to improve patient' access to emerging technologies."<sup>34</sup> Administrator Verma states that the Administration's vision is "to protect and secure Medicare and ensure beneficiaries have access to the latest medical technologies. The advent of novel medical technologies requires CMS to remove barriers to ensure safe and effective treatments are readily accessible to beneficiaries without delaying patient care. In essence, keeping new technologies and treatments moving from bench to bedside—and into the hands of those who need them most."<sup>35</sup>

ARM applauds these statements and looks forward to working with the Administration to implement the resulting policies. One policy that CMS could change to greatly improve access to novel medical technologies is the frequency of the NTAP. The current process provides for NTAPs to hospitals to occur only at the

---

<sup>32</sup> 84 Fed. Reg. 19174.

<sup>33</sup> *Id.*

<sup>34</sup> <https://www.cms.gov/newsroom/press-releases/speech-remarks-administrator-seema-verma-medical-device-manufacturers-association-annual-meeting>

<sup>35</sup> *Id.*

beginning of the fiscal year. ARM believes that this requirement unnecessarily delays access to innovative and often lifesaving therapies for Medicare beneficiaries. As such, ARM urges CMS to implement a more frequent NTAP approval process consistent with the Administrator's vision and other sites of care such as the hospital outpatient setting. Further, a more frequent NTAP would enhance the quality of data for the Agency to use for rate setting purposes. The Agency would, in theory, have more claims data associated with the new technology to analyze when establishing the next fiscal year's relative weights.

## **X. Conclusion**

In conclusion, ARM believes that the field of regenerative medicine has the potential to heal people and bend the health cost curve toward lower long-term costs and higher quality outcomes. This trend is already evidenced by several approved and marketed first-generation regenerative medicine products that are demonstrating both clinical and cost reduction value. Specifically, by reducing hospital care, the need for physician, clinical and professional services, nursing, and home healthcare, we could substantially reduce overall healthcare expenses. The ARM is confident that meaningful improvements in clinical outcomes and cost reduction can be accomplished through regenerative medicine technologies.

Much of the dialogue around healthcare in recent years has focused on the issues of broadening access (through insurance reforms) and controlling costs through Medicare and Medicaid reimbursement reforms such as payment cuts to health providers. Reducing expenditures alone will not enable us to improve clinical outcomes and achieve enhanced patient quality of life if it hampers innovation.

ARM supports the goals of NTAP. It is critical for CMS to develop and implement policies and programs that support the use of new technologies such as uniform add payment at 65 percent of the costs of the technology and utilizing acquisition cost in the outlier calculation. This is particularly true for regenerative medicine and other advanced therapies that hold the promise of durably treating and potentially even curing disease.

We thank the Agency for its many proposals and statements in the Proposed Rule and look forward to working with CMS to establish policies that promote appropriate access to regenerative medicine therapies in both the near term and long. Please free to contact me at [rfab@alliancerm.org](mailto:rfab@alliancerm.org) with questions.

Sincerely,



Robert J. Falb  
Director, U.S. Policy and Advocacy