



June 24, 2019

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

Sent electronically

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 (CMS-1716-P)

Dear Administrator Verma:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 230 institutions across the health care spectrum, appreciates the opportunity to submit comments regarding the Centers for Medicare & Medicaid Services (CMS)' *Medicare Hospital Inpatient Prospective Payment System (IPPS) Proposed Rule for FY 2020*.¹ While we recognize there are numerous important payment issues addressed in the proposed rule, PMC's comments are confined to the impact of specific proposed payment changes on beneficiary access to chimeric antigen receptor (CAR) T-cell therapies and cell and gene therapies like them that are forthcoming. PMC commends CMS for its efforts to ensure appropriate payment for CAR T-cell therapies through proposed updates for FY 2020, however we suggest additional considerations below that may lead CMS to the creation of a long-term solution for adequate payment of these and other highly specialized medicines.

Personalized medicine is an evolving field that uses diagnostic tools to identify specific biological markers, often genetic, to help determine which medical treatments and procedures will be best for each patient. By combining this information with an individual's medical history, circumstances, and values, personalized medicine allows doctors and patients to develop targeted prevention and treatment plans.

Personalized medicine is helping to shift the patient and provider experience away

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from trial-and-error and toward a more streamlined process for making clinical decisions, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction. As noted above, PMC's members are leading the way in personalized medicine and recommend that patients who may benefit from this approach undergo appropriate testing and tailored treatment as soon as possible during their clinical experiences.

PMC recently released a report titled *Personalized Medicine at FDA: A Progress and Outlook Report*. The report documents a record number of new personalized medicine approvals by the U.S. Food and Drug Administration (FDA) in 2018. This marks the fifth consecutive year that personalized medicines accounted for more than 20 percent of all new drug approvals.ⁱⁱ Innovation is at an all-time high, as reflected in the high number of personalized medicines documented in this report. The pipeline for significant cell and gene therapies is robust. A study conducted by the Massachusetts Institute of Technology NEWDIGS FoCUS Project predicts that by the end of 2022 there will be about 40 gene therapy products approved by the FDA, with 45 percent of these approvals being for products targeting cancer.ⁱⁱⁱ These therapies continue to pose challenges related to coverage and reimbursement and their implementation into clinical practice. Newer approaches to treatment, such as those with CAR T-cell therapies, represent a significant advancement in care for specific patient populations. We appreciate CMS' acknowledgement that the current inpatient system does not provide adequate payment for newer treatment approaches and that updates are critical in order to ensure delivery of treatments for Medicare beneficiaries that meet the highest standard of care. PMC's feedback on proposed changes in the proposed rule are intended to support CMS in providing beneficiary access to life-saving cell and gene therapies now and in the future.

Statement of Neutrality

Many of PMC's members will present their own responses to CMS and will actively advocate for those positions. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance, and are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to the Medicare *IPPS Proposed Rule* for FY 2020.

Challenges with Current Reimbursement Paradigm

PMC is committed to ensuring that coverage and reimbursement policies do not serve as a barrier to the timely and appropriate use of personalized treatment options by providers. We recognize that CMS did respond to concerns raised by PMC and other stakeholders regarding the FY 2019 *IPPS Proposed Rule*,^{iv} and that the agency attempted to identify ways to improve payment for cases involving CAR T-cell therapies and other new technologies. In the *IPPS Proposed Rule* for FY 2020, CMS acknowledges continued challenges unique therapies like CAR T-cell therapies pose to the current reimbursement paradigm.

The New Technology Add-On Payment (NTAP) policy used by CMS to provide additional payment for breakthrough technologies was not developed with cell and gene therapies, including CAR T-cell therapies and the small patient populations they serve, in mind. Even with an NTAP, hospitals still require a way to recover the acquisition costs of these therapies and the costs associated with their delivery in the short term and in the long term, after they are no longer eligible for NTAP payments.

We commend CMS for acknowledging that the existing NTAP rate is not providing enough incentives for the use of new treatments. Increases to the NTAP rate and updates to the determination criteria and frequency of adoption of NTAPs are central to ensuring the program adapts accordingly.

Payments under a Medicare Severity-Diagnosis Related Group (MS-DRG) are set with a two-year lag, so new technologies are often not included in payment calculations due to CMS' use of historical costs and charges. As a result, for a period of time, hospitals are not fully reimbursed for the use of innovative treatments and hospitals can be left with significant unreimbursed costs for patient care. Limited options are available for additional payment. Many therapies that qualify for an NTAP will be used broadly and replace the standard of care in a disease area. This would automatically update an existing MS-DRG, however other NTAP therapies will be used in a limited group of patients in a MS-DRG and highlight potential new MS-DRG assignment. Under the IPPS system there is no current policy for automatic data collection and case review that would lead to new MS-DRG assignment, after NTAP eligibility expires. PMC appreciated CMS' recognition in the *IPPS Proposed Rule* that existing MS-DRGs do not provide adequate payment for CAR T-cell therapies and other cell- and gene-based therapies.

The agency has demonstrated an openness to stakeholder feedback on how the reimbursement paradigm can be structured to ensure sufficient payments to cover the cost of care with cell and gene therapies. Further, we believe that with some modification, CMS' proposed payment updates to the current inpatient payment structure will serve to continue access to CAR T-cell and other innovative therapies in the future.

Considerations for CMS in Finalizing Proposed Changes

The *IPPS Proposed Rule* for FY 2020 signals a continued willingness at the agency to adapt the current payment structure to account for innovative treatments and proposes payment updates to the current inpatient payment structure that would continue access to CAR T-cell therapies and allow for improved access to cell and gene therapies in the future. PMC is pleased with the direction of the proposals put forward by CMS, but asks the agency's consideration of the following issues:

- The proposed changes to the NTAP payment rate — from the lesser of 50 percent of the costs of the new medical service or technology; or 50 percent of the amount by which the costs of the case exceed the standard MS-DRG payment to the lesser of 65 percent of the costs of the new medical service or technology; or 65 percent of the amount by which the costs of the case exceed the standard MS-DRG payment — are a critical step toward achieving a payment structure that can keep pace with the delivery of cell and gene therapies, but we are concerned that this payment rate is still insufficient to make hospitals whole. We support the payment increase but we believe CMS should use its regulatory authority to increase the NTAP amount more substantially. We recommend an increase to 80 percent of the cost of a therapy for NTAPs in general.
- As described above, some NTAP-eligible therapies will only be used in a subset of patients in a given MS-DRG and this use will identify potential new MS-DRG groupings. PMC recommends that CMS institute a policy to assess all technologies with an expiring

NTAP for new MS-DRG assignment. We envision a process similar to CMS' assignment of clinical ambulatory payment classifications (APCs) after sufficient data has been collected through a new technology. CMS should undertake the same level of review in the IPPS for cases using a cell or gene therapy when their NTAP expires.

- PMC supported the creation of a new MS-DRG code for CAR T-cell therapy in FY 2019. We understand that CMS did not propose the creation of a new MS-DRG in FY 2019 and believes that there is a lack of clinical and cost data to create a new MS-DRG for cases using CAR T-cell therapies in FY 2020. PMC believes that necessary data will be available and urges CMS to create a new MS-DRG in FY 2021. To facilitate data collection that will assist in future rate setting, hospitals should be encouraged to report on the voluntary value code 86 for CAR T-cell therapies, in addition to the mandatory new revenue code 0891 for Approved Cell Therapies in calculating NTAP and other payments. Additionally, during the data collection period, PMC urges the exclusion of clinical trial cases in the calculation of the MS-DRG as their costs vary widely because of the differing billing and charging practices, and they often disproportionately represent only the costs associated with delivery of these therapies. Clinical trial claims data is not representative of the average cost of the case for CAR T or any other services.
- In FY 2019, CMS raised the possibility of using a Cost to Charge Ratio (CCR) of 1.0 so that hospital charges for CAR T-cell treatment would be fully reflected in rate-setting and payment calculations. PMC generally supported exploration of this proposal in FY 2019 because it would help mitigate hospital losses. However, the proposal, which CMS put forward again in FY 2020, assumes that hospitals set charges equal to the cost of CAR T-cell therapies when, in reality, charging practices vary across hospitals. Use of a CCR of 1.0 with reported charges might overestimate costs for some hospitals. Rather than applying a CCR, calculating payments using the acquisition cost of CAR T-cell therapies reported with value code 86 may provide a more consistent method of recognizing hospitals' costs for CAR T-cell therapy and support collection of data described above.
- Lastly, the agency asked whether a disproportionate share hospital (DSH) and an indirect medical education (IME) adjustment should be made for cases assigned to any new MS-DRG for CAR T-cell therapy. These important adjustments are beneficial to patient care and medical training. The elimination of these add-on payments for CAR T-cell treatment would be unwise and PMC recommends that some level of assistance should continue for hospitals that rely upon DSH and IME payments to successfully treat the underserved populations and train physicians.

The potential changes in the FY 2020 *IPPS Proposed Rule* will significantly impact current FDA-approved CAR T-cell therapies and lay the foundation for a reimbursement pathway that will affect other novel cell and gene therapies as they are brought to market. PMC's goal is to protect innovation in the cell and gene therapy space and sustain access to personalized cell and gene-based therapies through payments that appropriately reflect the cost of delivering care. As stated in our comments on the FY 2019 *IPPS Proposed Rule*, PMC encourages CMS to adopt a pathway that creates flexibility in the adoption of future innovations into the IPPS reimbursement structure; enables providers and patients to select the most appropriate treatment

and site of care; and allows for continued refinement by all stakeholders to improve patient outcomes while appropriately managing costs.

Conclusion

Thank you for your leadership and for considering our comments. As CMS moves forward with finalizing the Medicare *IPPS Rule* for FY 2020, we urge you to ensure that the decisions made bring us closer to the goal PMC shares with the agency of delivering appropriate, efficient, and accessible health care to every patient. We welcome the opportunity to serve as a resource for you. If you have any questions about the content of this letter, please contact me at 202-589-1769 or cbens@personalizedmedicinecoalition.org.

Sincerely,



Cynthia A. Bens
Senior Vice President, Public Policy

ⁱ Centers for Medicare & Medicaid Services. *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 (CMS-1716-P)* May 3, 2019 <https://www.regulations.gov/document?D=CMS-2019-0073-0003>

ⁱⁱ Personalized Medicine Coalition. *Personalized Medicine at FDA: A Progress and Outlook Report*. February 13, 2019 [http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM at FDA A Progress and Outlook Report.pdf](http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM%20at%20FDA%20A%20Progress%20and%20Outlook%20Report.pdf)

ⁱⁱⁱ Massachusetts Institute of Technology NEWDIGS FoCUS Project. *Existing Gene Therapy Pipeline Likely to Yield Dozens of Approved Products Within Five Years; Research Brief*. November 13, 2017. http://newdigs.mit.edu/sites/default/files/FoCUS_Research_Brief_2017F211v011.pdf

^{iv} Personalized Medicine Coalition. *Comments on 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 2019 Rates; Proposed Quality Reporting Requirements for Specific Providers; Proposed Medicare and Medicaid Electronic Health Record (EHR) Incentive Programs (Promoting Interoperability Programs) Requirements for Eligible Hospitals, Critical Access Hospitals, and Eligible Professionals; Medicare Cost Reporting Requirements; and Physician Certification and Recertification of Claims [CMS-1694-P]*. June 25, 2018 http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_CMS_IPPS_2019.pdf