PERSONALIZED MEDICINE COALITION

June 9, 2023

Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attn: CMS–1785–P
7500 Security Boulevard
Baltimore, MD 21244-1850

Re: Medicare Program; Proposed Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals and the Long-Term Care Hospital Prospective Payment System and Policy Changes and Fiscal Year 2024 Rates; etc (CMS-2023-0057-0003)

Dear Administrator Brooks-LaSure:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 220 institutions from across the health care spectrum, thanks the Centers for Medicare & Medicaid Services (CMS) for the opportunity to submit comments on the *Medicare Hospital Inpatient Prospective Payment System (IPPS) Proposed Rule for FY 2024*.[i] As you may recall, in its comment letter on CMS' *IPPS Proposed Rule for FY 2021*, PMC supported the establishment of a new Medicare Severity-Diagnosis Related Group (MS-DRG) for chimeric antigen receptor (CAR) T-cell therapies as a way to accelerate access to these potentially life-saving personalized treatments.[ii] We believe the thoughtful continuation of MS-DRG 018 as outlined in CMS' proposed rule for FY 2024 will continue to yield significant benefits for patients, providers, and hospitals, and we applaud CMS for taking this approach. PMC is concerned, however, that the documentation requirements and timeline changes CMS is proposing for New Technology Add-On Payment (NTAP) applications could reduce patient access to new and innovative medical technologies like CAR T-cell therapies. While PMC recognizes there are numerous important payment issues addressed in the *IPPS Proposed Rule for FY 2024*, our comments are limited to the impact of specific proposed policy changes on beneficiary access to CAR T-cell therapies and other transformative personalized medicine technologies forthcoming in cancer and other diseases.

PMC defines personalized medicine as an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients.

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Personalized medicine is helping to shift the patient and provider experiences away from trial-and-error 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. 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.

CAR T-cell therapy represents a significant advancement in personalized medicine. Some cancer patients with very poor prognoses have experienced life-improving and life-extending outcomes resulting from CAR T-cell therapy. The CAR T-cell therapies already on the market have had a profound impact on the lives of patients with certain forms of lymphoma, leukemia, and multiple myeloma. Dr. Carl June, the University of Pennsylvania immunologist who designed the first CAR T-cell treatment, has stated that "We can now conclude that CAR T-cells can actually cure patients" based on evidence that CAR T-cells are still active in patients a decade after treatment and the fact that at least two patients remain free of cancer.[iii] With CAR T-cell therapies being tested in hundreds of clinical trials, results like these and the promise of future cell and gene therapies provide hope for many patients with cancer and other hard-to-treat diseases.

Statement of Neutrality

Many of PMC's members will present their own responses to the *Medicare IPPS Proposed Rule for FY 2024* 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 proposed rule.

Considerations for CMS in Finalizing Proposed Rule

PMC appreciates that CMS' *IPPS Proposed Rule for FY 2024* takes a similar overall approach to the policies adopted for MS-DRG 018 as in previous years, which continue to help support greater patient access to CAR T-cell therapy. The FY 2024 proposed rule continues to provide a base payment rate that is the highest of the MS-DRGs now in place, demonstrating a continued willingness at the agency to maintain access to CAR T-cell therapies. We believe the FY 2024 proposal is largely responsive to PMC's previous requests for a permanent reimbursement solution for CAR T-cell therapy that is formulated in a manner reflecting the true expenses associated with patient care.[iv]

We continue to be concerned, however, for the long-term viability of MS-DRG 018 as more novel products enter the market. If CMS were to assign new, higher volume, lower cost therapies to MS-DRG 018, it could potentially distort the relative weight of the MS-DRG and under-reimburse CAR T-cell therapies. We share the agency's interest in developing a sustainable mechanism to accommodate the expanding portfolio of transformative therapies for which providers will need adequate reimbursement. Since CMS' FY 2022 policy that expanded MS-DRG 018 to include certain other immunotherapies, however, we have cautioned that the inclusion of additional procedure codes associated with other therapies could lead to reductions in the base rate for MS-DRG 018 over time.[v] We encourage CMS to clarify its methodology for the inclusion of new procedure codes within MS-DRG 018. We also ask CMS

to continue assessing the appropriateness of therapies assigned to MS-DRG 018 so that the cost and resource needs of potential new additions to MS-DRG 018 do not harm access to current therapies.

New Technology Add-on Payment (NTAP)s encourage hospitals to adopt breakthrough technologies by helping them recover some of the increased costs associated with offering innovative treatments to patients. In our comments to CMS on previous IPPS proposed rules, PMC has asked CMS to grant NTAP status to CAR T-cell therapies and consider how new CAR T-cell therapies in the research and development pipeline differ from the CAR T-cell treatments now available, with differences relating to the uniqueness of patient populations, disease areas treated, specific antigen targets, and other differences in the therapies themselves. NTAP status provides another way CMS can remove a potential barrier to CAR-T cell therapy access when considering these differences. While there are currently no CAR T-cell therapies with NTAP applications, we understand that two applications for NTAP status included in the FY 2024 proposed rule related to personalized medicine beyond MS-DRG 018 include a targeted therapy and biomarker detection tool. We encourage CMS to assign NTAP status for new treatments and technologies supporting personalized medicine that meet the required criteria, including the applications under consideration for VANFLYTA® (quizartinib) and CYTALUX® (pafolacianine). Doing so will remove a potential barrier to accessing innovative treatments and tools advancing this approach to care.

Since providers rely on NTAP to be able to provide access to new and innovative treatments and technologies, we are concerned that CMS's proposed requirements for NTAP application and timeline changes could have significant implications for the availability and duration of add-on payments and, thus, harm patient access. NTAP status lasts three years if the three-year anniversary date of Food and Drug Administration (FDA) approval is in the second half of the fiscal year (i.e., after April 1); otherwise, the duration is only two years. By moving the FDA approval deadline for NTAP eligibility up by two months, from July 1 to May 1, CMS' proposed change will reduce the number of technologies eligible for three full years of NTAP status. A product launching on May 2, for example, would not receive an NTAP until October of the following year, creating reimbursement uncertainty for providers over an extended period with potential negative effects on the product's uptake.[vi] Reducing the duration of NTAPs and the timeliness of reimbursement adequacy could reduce patient access to future CAR T-cell therapies and other important technologies advancing personalized medicine. Therefore, we encourage CMS to maintain the existing timeline. We would also support CMS increasing the number of NTAP submission periods to align NTAP reimbursement more closely with FDA marketing authorization and facilitate more timely patient access to innovative treatments.

In addition, CMS is proposing to require an NTAP applicant to have a "complete and active" FDA market authorization request at the time the NTAP application is submitted. Since applications are already being developed for FY 2025, requiring applicants to have submitted their FDA market authorization requests by the time they apply for NTAP this fall could decrease the technologies otherwise eligible for an NTAP in FY 2025, potentially reducing patients' access to important new technologies. Furthermore, because this "complete and active" criterion is not defined elsewhere in statute, we believe this language must be flexible to accommodate drugs, devices, and diagnostics eligible for NTAP across different application types and regulatory approval pathways at FDA where approval timelines and processes may differ, such as through rolling review, accelerated approval, or the real-time oncology review (RTOR) program. For example, rolling review is available for original

Biologic License Application (BLA) or New Drug Application (NDA) applications that have been awarded breakthrough therapy or fast track designations or are subject to real-time oncology review or accelerated approval. By requiring a "complete and active" marketing authorization request at the time of NTAP application submission, CMS would prevent a manufacturer from being able to apply for NTAP for a product that has initiated rolling review or RTOR but does not yet have a completed BLA or NDA with the FDA. Since the timing of the BLA or NDA completion can already cause delays between FDA marketing authorization and NTAP reimbursement, we encourage CMS to clarify this language to ensure the gamut of personalized medicine treatments and technologies remain eligible for NTAP and can reach the patients that need them, without creating further delays in the availability of NTAP status.

Finally, in our FY 2023 comment letter, PMC applauded the agency for including a request for information calling attention to the special challenges of reimbursement adequacy often faced for rare disease treatments under the IPPS.[vii] We encourage the agency to continue to take steps toward meaningful changes that reduce IPPS payment disparities for treatment of rare disease patients. We also applaud CMS' commitment in the FY 2024 proposed rule to advancing health equity-related measures and to expand the collection, reporting, and analysis of standardized health equity data, such as data on social determinants of health, as part of the *CMS Framework for Health Equity 2022-2032*.[viii] We appreciate CMS' willingness to solicit feedback in this proposed rule on how it can improve its use and breadth of health equity measures. As the agency considers next steps for advancing health equity, we encourage CMS to incorporate the perspectives of patients and providers from diverse backgrounds and underserved communities to facilitate a greater understanding of how CMS can ultimately support robust and equitable patient access to CAR T-cell therapies and other transformative personalized medicine treatments or technologies.

PMC appreciates your commitment to ensuring that beneficiaries have access to transformative therapies. We look forward to working with you and your colleagues at CMS to protect patient access to CAR T-cell therapy and to continue fostering innovation in this and related therapeutic areas for patients with unmet needs. If you have any questions about the content of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

[i] Centers for Medicare & Medicaid Services. *Medicare Program; Proposed Hospital Inpatient Prospective Payment Systems for Acute Care Hospitals; etc. (CMS-2023-0057-0003).* May 1, 2023. https://www.regulations.gov/document/CMS-2023-0057-0003. (Accessed June 5, 2023.)

[ii] Personalized Medicine Coalition. *Comment Letter on 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 2021 Rates; Quality Reporting and Medicare and Medicaid*

Promoting Interoperability Programs Requirements for Eligible Hospitals and Critical Access Hospitals. July 10, 2020. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_CAR-T_MS-DRG_7.10.20.pdf. (Accessed June 5, 2023.)

[iii] Mast, Jason. "Carl June: 'We can now conclude that CAR-T cells can actually cure patients.'" *Endpoints*. February 2, 2022. https://endpts.com/carl-june-we-can-now-conclude-that-car-t-cells-can-actually-cure-patients/. (Accessed June 5, 2023.)

[iv] Personalized Medicine Coalition. *Letter to Administrator Verma on Reimbursement for Chimeric Antigen Receptor (CAR) T-cell Therapy*. April 22, 2020. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_on_CMS_Reimbursement_Policy_for_CAR_T-cell_Therapy_April_2020.pdf. (Accessed June 5, 2023.)

[v] Personalized Medicine Coalition. *Comment Letter on 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 2022 Rates; Quality Reporting and Medicare and Medicaid Promoting Interoperability Programs Requirements for Eligible Hospitals and Critical Access Hospitals*. June 28, 2021. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_Comments_CAR-T_MSDRG_IPPS_FY_2022.pdf. (Accessed June 5, 2023.)

[vi] Gustafson, Kolton et al. "CAR-T Reimbursement Updates Proposed for FY2024." *Avalere*. May 4, 2023. https://avalere.com/insights/car-t-reimbursement-updates. (Accessed June 5, 2023.)

[vii] Personalized Medicine Coalition. *Comment Letter on Medicare Program; Fiscal Year 2023 Hospital Inpatient Prospective Payment Systems; Quality Programs and Medicare Promoting Interoperability Program Requirements, etc*. June 17, 2022. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_comment_letter_FY_2023_IPPS_proposed_rule.pdf. (Accessed June 5, 2023.)

[viii] Centers for Medicare & Medicaid Services. *CMS Framework for Health Equity 2022-2032*. April 2022. https://www.cms.gov/files/document/cms-framework-health-equity.pdf. (Accessed June 5, 2023.)