DEPARTMENT OF HEALTH AND HUMAN SERVICES

Medicare Program; Virtual Meeting of the Medicare Evidence Development and Coverage Advisory Committee—December 7, 2022

AGENCY: Centers for Medicare & Medicaid Services (CMS), Health and Human Services (HHS). MEDCAC Meeting

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RE: CIBMTR and ASTCT Comments

The Center for International Blood and Marrow Transplant Research (CIBMTR) is a clinical research program dedicated to addressing important issues in the field of Hematopoietic Cell Transplantation (HCT) and Adoptive Cell Therapy (ACT). CIBMTR maintains a large outcomes registry of real-world data with information for >625,000 HCT/ACT patients in treatments centers in the United States (US) and globally and provides statistical support and scientific expertise for analyzing those data. The CIBMTR registry data is routinely used to fill in knowledge gaps where randomized trials were not feasible.

Many patients with specific diseases and/or at certain ages are denied access to cellular therapy in the US due lack of insurance coverage by the Centers for Medicare and Medicaid Services (Medicare). To help secure Medicare coverage for these patients, CIBMTR, the National Marrow Donor Program (NMDP) and The American Society of Transplant and Cellular Therapy (ASTCT), along with other organizations partnered with Medicare to launch CED studies. These CED studies allow Medicare to provide coverage to patients enrolled on the clinical studies that inform policy decisions. Recognizing the importance of the Medicare CED process, CIBMTR has been engaged in Medicare CED studies since 2010 and is currently supporting 5 studies focused on myelodysplastic syndrome, myelofibrosis, and multiple myeloma for patients older than 65 and sickle cell disease for adolescents and adults; 7,295 patients have received transplants with Medicare reimbursement because of these studies.

Given CIBMTR's hands-on experience with the design, development, and implementation of Medicare CED studies, we respectfully provide the following comments:

- *We advocate for studies that are straightforward, transparent, and timely: Study design should support timely access to the population of interest, use of current technologies and clinical frameworks, and relevant and timely policy decisions. We ask for consideration of a mechanism for early dialogue about proposed CED language that includes key stakeholders, such as CIBMTR, to help mitigate these potential issues.*
  While, overall, the CIBMTR Medicare CED study portfolio has successfully supported study intent, there is significant variability between studies. We have found that CED studies that require more exclusive inclusion criteria and complex specificity of approach have both lengthened the time for and decreased the total numbers of patients eligible for accrual; significantly slowing analysis of results needed for policy decision making. For example, the design of the Multiple Myeloma study significantly impacted the ability to accrue patients where currently there are only 27 patients enrolled for a study that opened for enrollment in July 2017. With rapid changes to cellular therapy science, CEDs should be written so that research studies can accommodate these changing therapies. In particular, the inclusion of particular disease

scoring systems within CED study requirements can become problematic as the scoring system may evolve or become clinically obsolete during the timeframe in which the CED is open.

- *It is important to recognize that these studies have no source of funding. Clinical studies are costly, and the lack of a funding mechanism represents a real limitation to their fulfillment.* Since the launch of the first CED study, CIBMTR modified its existing registry infrastructure in multiple ways to support each CED study. These modifications include updates to data systems necessary to collect, store and extract detailed data to support each CED study, development of systems supporting necessary study eligibility confirmation to centers allowing access to reimbursement, and updates to patient consent so that participation in the CED study does not assume participation in other CIBMTR activities. Finally, CIBMTR trains staff members specifically for use of the CED study infrastructure and effective management of ongoing studies. Because funding support for these studies is not available, the cost of infrastructure modifications as well as staff management support for ongoing studies is absorbed by CIBMTR. Due to the timeframe of interest to CMS in relation to HCT outcomes, the MDS CED has been open since 2010, which means these costs have been absorbed by the CIBMTR and the clinical treatment community for 12 years. Over time, this is not a sustainable approach for this critical work.

- *There is a need for additional clarity around the translation of study results into a National Coverage Determination (NCD), clarification of what evidence is needed to effect policy change and, using study results, a transition plan bridging the time between a study and a final NCD.* A CED study plan will have both a start and end date, along with a defined population type and enrollment necessary for its statistical analysis plan. There are three time periods during which beneficiaries face a strong potential for lack of coverage during a National Coverage Analysis involving a CED: 1) during the initial NCA process, which takes between 9-12 months, 2) during the time-period between reaching the study accrual target and either an extension or additional CED-compliant study being approved, and 3) during the 9-12 month reconsideration time-period at the end of a CED. For individuals needing HCT to treat their disease, these gaps in coverage are life-threatening – patients that are candidates for HCT may have very limited treatment options to bridge these time periods.
  Patients are accrued to the study population in advance of the final analysis and the number of patients necessary for the analysis is statistically predetermined. Therefore, at some point, study enrollment is met, and no additional patients are required, creating a situation where there is a gap in coverage for those patients who are study eligible but can no longer accrue to the population being formally analyzed. The CIBMTR has had to extend the current MDS study multiple times in order to allow for continued access during the required 5-year post-transplant analysis period for the study group; without this, 2416 Medicare eligible patients of the 6887 treated for MDS would have been ineligible for treatment during the CED period. We propose, as a standard practice, that CMS: a) mandate that the Medicare Administrative Contractors (MACs) allow non-study coverage during both the initial and reconsideration National Coverage Analyses periods and b) create CED study language to accommodate overall study accrual to occur until the time a NCD is in place, or a decision is made to no longer provide coverage.

As such, CMS should have a clear, streamlined pathway to review evidence generated under CED, and other evidence available in the medical literature to arrive at an expedient updated NCD using CED study results.

- *There is a need for communication tools to help centers and patients better understand billing and reimbursement under CED.*
  CMS should develop better communication tools to help centers who treat patients under CED to better align billing codes to ensure appropriate reimbursement for care delivered under CED. Centers participating in CIBMTR CED studies and enrolling patients have had substantial difficulty navigating the billing system, which may limit access for patients in certain health systems if there is a lack of confidence in reimbursement despite CED.