Jeffrey Chell, M.D.
Chief Executive Officer
National Marrow Donor Program, Be The Match
500 N. 5th Street
Minneapolis, MN 55401

Tamara Syrek-Jensen
Director, Coverage and Analysis Group
Centers for Medicare and Medicaid Services
Office of Clinical Standards and Quality
Coverage and Analysis Group
7500 Security Boulevard, Mail Stop C1-09-06
Baltimore, Maryland 21244-1850

November 23, 2015

The National Marrow Donor Program (NMDP) is the world's leading nonprofit organization focused on saving lives through blood, marrow and cord blood hematopoietic cell transplantation (HCT). The NMDP operates the Be The Match® registry, the largest and most diverse donor registry in the world, through a competed contract overseen by the Health Resources and Services Administration (HRSA) Division of Transplantation. In addition to operating the registry, the NMDP holds the HRSA contract for the Office of Patient Advocacy/Single Point of Access and is responsible for assisting patients facing barriers to transplant. Through this congressionally-established office, the NMDP helps patients navigate the complexities of the health care system, with a primary focus on insurance and financial barriers that prevent patients from receiving a transplant.

The limitations of Medicare coverage for HCT is the single greatest insurance barrier faced by the patients we serve. Because of the age-related onset of most hematologic malignancies, many of the individuals seeking transplantation are Medicare beneficiaries. As operators of the HRSA OPA/SPA contract, and as an organization whose mission is to deliver cures for blood cancers, we continue to actively pursue significant improvements to access to HCT on behalf of our Medicare beneficiary stakeholders. **We very much appreciate CMS's partnership in finding a way to provide these patients with the treatment options they are seeking.**

We understand and appreciate that CMS is trying to provide a pathway to coverage for allogeneic HCT for myelofibrosis, multiple myeloma and sickle cell disease through its proposal for the use of Coverage with Evidence Development (CED). CED is a framework that has been working successfully for myelodysplastic syndrome (MDS) since 2010. While there are additional data and reporting requirements associated with this coverage structure, over 1400 patients that previously had no access to HCT as a therapy for their condition have received a transplant. However, the study requirements proposed for myelofibrosis, multiple myeloma and sickle cell disease are very different than the model that has been used successfully for MDS.

The proposed requirement for concurrent non-HCT controls, without also having a parallel observational study arm, will greatly limit the access that most beneficiaries will have to HCT, despite CMS's positive intentions. The American College of Surgeons Commission on Cancer has accredited over 1500 cancer

hospitals in the United States[1]. However, HCT is a highly-specialized medical field; allogeneic transplantation is performed in approximately 130 adult transplant programs in the United States, most within major academic medical centers[2]. Of these 130 allogenic transplant centers, 105 are actively participating in the observational study for MDS, while only 34 centers are able to allocate enough research resources to participate in the comparative clinical trial. To clarify – the MDS comparative clinical trial is open in approximately one-quarter (25%) of the allogenic transplant centers in the country and approximately 2% of the Commission of Cancer Accredited facilities in the United States.

Application of the study design as proposed by CMS will result in unequal access to care for its beneficiaries based on where they reside. We expect that the number of centers that would be able open studies that satisfy the current proposed requirements in the current National Coverage Analysis to be even fewer than those participating in the MDS comparative trial, due to decreased numbers of affected patients in any particular geographic area in conjunction with the increased comparative data requirements. As Medicare does not provide travel and lodging relocation benefits to beneficiaries, many will be unable to seek treatment due to the lack of a participating hospital in their area. Due to the access issues described, we strongly object to CMS's proposed requirement for concurrent non-HCT controls and ask CMS to remove this requirement from the final coverage decision.

We understand the need for CMS to have data on low volume conditions, particularly those that will be new or expanded services to the beneficiary population. However, CMS's concern that clinically established outcomes will differ in the beneficiary population due to age, and its proposed corresponding study requirements to demonstrate this relationship, is likely unsupported. As part of the MDS CED CIBMTR study, interim analyses are reported to CMS on a regular basis. These analyses have all demonstrated that there are no statistically significant differences in outcomes between patients age 55-64 and patients age 65 and older[3]. Due to the age-associated development of hematologic malignancies, the affected population naturally clusters around the age of transition to Medicare. The median age of diagnosis for multiple myeloma is 69[4] and for myelofibrosis, the median age of diagnosis is 67 years[5] . Commercial insurance carriers routinely provide coverage for transplantation for these disease indications[6] and this coverage does not have age requirements associated with it. As these payers provide coverage for members up to their 65th birthday, the Medicare population of transplant recipients are not substantially older than those who receive transplant coverage under commercial policies.

Medicare's coverage of HCT for expanded indications will not drive inappropriate use of transplant in beneficiaries that are clinically inappropriate. Transplant is a clinically intensive procedure and the physicians that provide this care treat it accordingly; it is not provided as a 'last-chance' effort for all patients that fail other therapies. The Stem Cell Therapeutic and Research Act (reauthorized in 2010)

---

[1] American College of Surgeons Commission on Cancer program

[2] FY2016 CMS MedPar Data

[3] Attalah, Ehab, Outcome of Patients 65 Years and Older with Myelodysplastic Syndrome (MDS) Receiving Allogeneic Hematopoietic Stem Cell Transplantation Compared to Patients 55-64 Years of Age, https://ash.confex.com/ash/2015/webprogram/Paper78833.html; McClune BL, J Clin Oncol. 2010 Apr 10;28(11):1878-87; Deeg, HJ, Biol Blood Marrow Transplant. 2015 Nov;21(11):1883-7. doi: 10.1016/j.bbmt.2015.09.005. Epub 2015 Sep 11;

[4] National Institutes for Health, National Cancer Institute SEER data for Myeloma

[5] Deadmond MA, Smith-Gagen JA. Changing incidence of myeloproliferative neoplasms: trends and subgroup risk profiles in the USA 1973-2011. J Cancer Res Clin Oncol. 2015 [Epub ahead of print; 2015 May 13].

[6] NMDP analysis of publicly available commercial insurance policies, submitted to CMS in January 2015.

requires that all providers submit data on each allogeneic transplant that they provide, including long-term follow-up data. **Each center receives an aggregate score based on the overall survival data for *all patients* treated by the center – there are no exclusions for particular patient categories or age ranges.** This score is utilized by commercial payers to assess transplant program performance and, subsequently, appropriateness for inclusion in their provider networks. This system inherently motivates physicians to apply evidence-based clinical patient eligibility criteria to those seeking care.

We understand that CMS needs to carefully consider the coverage policies it applies to the beneficiary population and we recognize that CMS is attempting to expand coverage in a way that will positively impact beneficiaries affected by these illnesses. We are willing to fulfill the administrative requirements of CED, but ask that CMS understand the needs of its beneficiary population and the unequal access conditions it will create for beneficiaries by applying the currently proposed study requirements. We are eager to assist in efforts to improve access for our patient stakeholders. Please contact Stephanie Farnia (612-884-8640, sfarnia@ndmp.org) with any additional questions on this issue.

Sincerely,

Jeffrey Chell, M.D.

Chief Executive Officer
National Marrow Donor Program, Be The Match