November 4, 2015

On October 29, the Centers for Medicare and Medicaid Services (CMS) issued a proposed decision memo regarding Medicare coverage for allogeneic hematopoietic cell transplant (HCT) for three indications: Multiple Myeloma, Sickle Cell Disease and Myelofibrosis. The proposed decision memo is in response to a formal request made by the ASBMT and NMDP/Be The Match in January 2015, with the intent of expanding Medicare's coverage of HCT for a limited set of diseases.

However, some of the proposed requirements may actually decrease access to HCT. We need your help to tell CMS that it is important to find a reasonable way to conduct this research. Please submit a comment to CMS.

Coverage with Evidence Development

Medicare has proposed Coverage with Evidence Development (CED), which means that Medicare beneficiaries have coverage for transplant if they participate in an approved clinical study. This is the same mechanism currently in place for Medicare's coverage of Myelodysplastic Syndromes. While coverage within a study does create additional tracking and reporting burdens, this mechanism has worked successfully for MDS and has allowed over 1400 patients with MDS to receive HCT.

While we greatly appreciate CMS's intent to provide a pathway to coverage for these three diseases through clinical studies, the proposed study requirements for SCD, Multiple Myeloma and Myelofibrosis *are different* than those required in the MDS study and will be extremely difficult to satisfy by the transplant community.

At the current time, we feel that the study structures being proposed are prohibitive due to a lack of understanding on the part of CMS staff and do not stem from a deliberate intention to block access to HCT.

Issue of Concern: Concurrent non-HCT Controls

The primary issue of concern with the proposed decision is with CMS's requirement for ***concurrent non-HCT controls***. Concurrent non-HCT controls are problematic for the following reasons:

- **Requiring concurrent non-HCT controls which will severely hinder our ability to accrue patients**. Concurrent controls are highly problematic for these indications, due to their rarity and severity. Most patients will be seeking any and all curative options for their illness, making it unlikely for them to consent to participation in a non-HCT control group. Biologic assignment trials in allogeneic HCT are less feasible and less relevant as alternative donor sources are increasingly accepted by the transplant community. Patients who have other factors that render them ineligible for the procedure are likely to be poor controls and will not provide CMS with the comparative analysis that they seek. In the case of Myelofibrosis, those remaining patients that are HCT-eligible but do not elect to pursue transplant will likely be a very small subset within an already limited population.

- **CMS is interested in long-term survival and quality-of-life outcomes**. The timeline to accumulate sufficient numbers of patients into these studies and to evaluate the long-term endpoints of interest to CMS will be very lengthy – at least 5-10 years, if not 20 years. We support Medicare's interest in understanding the late consequences of the procedure by developing long-term follow-up data on a sufficient number of transplant recipients. However, due to this extended timeframe, we need to ensure that patients have wide access to these studies and that the studies are not structured in a way that limits participation to a small group of patients or centers. Broad access to these trials is

essential due to ethical considerations in being able to offer the only current potentially curative therapy available to patients with a known poor prognosis.

- **The non-HCT outcomes of these illnesses are well-known.** These are indications that prove fatal within short timeframes or, in the case of SCD, cause significant morbidity and pain. We understand CMS's need to demonstrate effectiveness of a therapy in their particular beneficiary groups (age 65+ or those who are disabled), but further documentation of well-established and severe or fatal clinical outcomes are unnecessary.

- **These indications are rare diseases that do not fit CMS's usual model for clinical study designs.** The vast majority of the coverage decisions CMS makes are related to drugs and devices, many of which are utilized in clinical indications with substantially higher incidence and prevalence rates than those affecting transplant patients. CMS should be encouraged to consider alternate frameworks for rare diseases and low-volume procedures of curative intent, similar to the Breakthrough and Accelerated Approval Processes utilized by the U.S. Food and Drug Administration for orphan conditions.

Our Proposed Alternative

The NMDP/Be The Match and ASBMT will be asking CMS to remove the requirement for concurrent non-HCT controls. We are evaluating options to propose as alternatives, including observational study designs, utilizing linkages between the CIBMTR registry and other databases and using identified non-concurrent cohort data sets developed in other trials. We welcome your input on this issue.

How you can help: Submit a Comment by November 28

Submit a comment to CMS. We need the transplant, biomedical and lay community to share their comments with CMS. Your thoughts on these issues are crucial, both for these particular indications and for establishing precedent for how future transplant indications will be evaluated. We also need you to reach out to patients and others, such as disease-specific advocacy groups, who have a stake in this decision.

CMS is accepting comments on their proposal for 30 days only; the comment period closes on **November 28, 2015**.

See guidance on how to submit a comment. This document can be shared widely with colleagues and interested stakeholders. **Please make a particular effort to connect with Government Relations or Federal Affairs contacts at your organizations.** Many of those individuals are familiar with Medicare processes and may be able to help augment or guide your efforts.

We will remain in contact as more information becomes available.NMDP/Be The Match and ASBMT are working together on a comprehensive and detailed response letter to CMS. Given the short window for comments, we are asking that you submit your responses in advance of our formal letter.

At the end of the 30-day public comment period, CMS has up to 60 days to review comments and formalize their decision about coverage. We expect a final decision by CMS in January 2016.

Thank you for your assistance on this very important effort.

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