



Advancing Transfusion and
Cellular Therapies Worldwide

November 25, 2015

Tamara Syrek Jensen, JD
Director, Coverage and Analysis Group
Centers for Medicare and Medicaid Services
7500 Security Blvd., Mail Stop S3-02-01
Baltimore, MD 21244-1850

Re: Proposed Decision Memo for Stem Cell Transplantation (Multiple Myeloma, Myelofibrosis, and Sickle Cell Disease (CAG-00444R))

Dear Ms. Jensen:

On behalf of the thousands of individual and institutional members involved in cellular therapies that we represent, AABB would like to provide comments and feedback regarding the Centers for Medicare and Medicaid Services' (CMS) recent proposed decision to modify the existing National Coverage Determination to expand coverage for allogeneic hematopoietic stem cell transplantation (HSCT) for multiple myeloma, myelofibrosis and sickle cell disease (SCD).

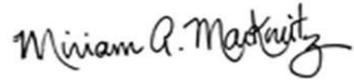
AABB applauds CMS' effort to open an important door for coverage for our nation's Medicare population. However, we are very concerned that the proposal, as written, would severely hinder patient access to potentially life-saving transplants by setting overly stringent and burdensome requirements for covered eligible trials. The proposed decision states that the coverage for HSCT would be allowed for these indications only through an approved study including a concurrent control group and focusing primarily on survival.

Allogeneic HSCT is currently the only potentially curative therapeutic option available for these diseases and, as such, it may be very difficult to enroll concurrent non-transplant controls. Patients seeking a curative option may be unwilling to be randomized to the control arm or be followed as a member of a non-transplant cohort. Moreover, studies of HSCT focusing primarily on survival in the short term (2-3 years) may have difficulty demonstrating a survival benefit for allogeneic HSCT, especially in patients with diseases like SCD, which is a chronic disease characterized by progressive organ damage, a very poor quality of life and shortened lifespan. SCD patients receiving transplants may demonstrate inferior short-term survival but then experience superior survival results in the long-term and enjoy a better quality of life.

It should also be noted that matched sibling donor transplantation in children with severe SCD results in >90% disease-free survival and can be considered a standard-of-care therapeutic option for these patients. If third party payers and state Medicaid programs follow the CMS guidelines, the proposed CMS decision will create a barrier to transplant access for this patient population.

AABB strongly urges CMS to amend its proposed requirements for study parameters, including removing the unnecessary use of concurrent controls. More reasonable study parameters should be put in place to allow for both greater patient access to life-saving transplants and valuable evaluation of this therapy.

Sincerely,

A handwritten signature in black ink that reads "Miriam A. Markowitz". The signature is written in a cursive, flowing style.

Miriam A. Markowitz
Chief Executive Officer