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**2017**

**President**

Kenneth Anderson, MD  
Dana-Farber Cancer Institute  
450 Brookline Avenue, Room M557  
Boston, MA 02115-5450

phone 617-632-2144

fax 617-632-2140

kenneth\_anderson@dfci.harvard.edu

**President-Elect**

Alexis Thompson, MD, MPH  
Ann & Robert H. Lurie Children's Hospital of Chicago  
225 E. Chicago Avenue  
Box #30  
Chicago, IL 60611

phone 312-227-4384

a-thompson@northwestern.edu

**Vice President**

Roy L. Silverstein, MD  
Medical College of Wisconsin  
Clinical Cancer Center  
9200 W. Wausconsin Avenue  
Milwaukee, WI 53226

phone 414-805-0815

rsilverstein@mcw.edu

**Secretary**

Robert A. Brodsky, MD  
Johns Hopkins University  
Ross Building, Room 1025  
720 Rutland Avenue  
Baltimore, MD 21205

phone 410-502-2546

brodso@jhmi.edu

**Treasurer**

Susan Shurin, MD  
222 Quince Street  
Unit 2C  
San Diego, CA 92103

phone 240-328-8542

sxs15@yahoo.com

**Councillors**

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**Executive Director**

Martha Liggett, Esq.  
mliggett@hematology.org

Cynthia B. Jones

Director

Department of Medical Assistance Services

600 East Broad Street, Suite 1300

Richmond, VA 23219

Dear Ms. Jones:

I am writing on behalf of the American Society of Hematology (ASH) regarding an issue recently brought to our attention by the American Society for Blood and Marrow Transplant (ASBMT), the National Marrow Donor Program (NMDP), and the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) concerning coverage for bone marrow transplantation for patients.

ASH represents over 17,000 clinicians and scientists worldwide, who are committed to the study and treatment of blood and blood-related diseases. These disorders encompass malignant hematologic disorders such as leukemia, lymphoma, and multiple myeloma, as well as non-malignant conditions such as sickle cell anemia, thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. In addition, hematologists were pioneers in demonstrating the potential of treating various hematologic diseases; and we continue to be innovators in the field of stem cell biology, bone marrow transplantation, regenerative medicine, transfusion medicine, and gene therapy. ASH membership is comprised of basic, translational, and clinical scientists, as well as physicians providing care to patients in diverse settings including teaching and community hospitals, as well as private practice.

The BMT CTN is a National Institutes of Health funded network established in 2001 to study transplantation for malignant and non-malignant hematologic diseases with the intent that multiple sites in the US would participate in this network to advance care of these patients. BMT CTN was recently contacted by a Virginia hospital which is an active clinical site for BMT CTN 1503: *A Study to Compare Bone Marrow Transplantation to Standard of Care in Adolescents and Young Adults with Severe Sickle Cell Disease*. The hospital cited denial from the Virginia Department of Medical Assistance Services (DMAS) of the transplantation authorization request on the grounds that transplant was considered an "experimental/investigational" treatment due to the therapy's provision within a clinical trial setting. However, bone marrow transplantation is not experimental or investigational for the treatment of sickle cell disease regardless of its inclusion in a clinical trial. Rather, of the three treatment options for patients living with sickle cell disease, it is the only curative option available at this time.

BMT CTN 1503 is a study to compare survival and disease-related outcomes between *two treatment options that are both considered to be standard of care; neither treatment is experimental or investigational practice*. This study is the first ever to compare survival between two accepted treatment options in a rigorous manner. The study is designed such that adolescents and young adults with severe sickle cell disease are assigned to their treatment arms based on availability of an HLA-matched related or unrelated donor. Those

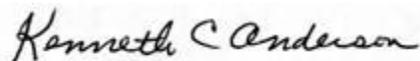
with a suitable donor are assigned to the donor arm and expected to undergo bone marrow transplant. Those without a donor are assigned to the no donor arm and expected to continue hydroxyurea, regular red blood cell transfusion, and pain management.

The BMT CTN 1503 study was developed upon completion of a phase II trial for bone marrow transplantation that used an identical transplant conditioning strategy (i.e. similar inclusion criteria, transplant conditioning regimen and graft-versus-host disease prophylaxis regimen). Additionally, there were 405 transplants for sickle cell disease in the United States for the period 2013 – 2015 that were reported to the Center for International Blood and Marrow Transplant Research, which collects data on all allogeneic transplants performed in the United States. A 2016 publication included a study of 1,000 recipients of HLA-identical sibling transplants performed between 1986 and 2013.<sup>1</sup> Furthermore, another 2016 report focused on HLA-matched adult unrelated donors, as only 18 percent of patients with sickle cell disease have an HLA-matched sibling donor in the United States.<sup>2</sup> The referenced data and studies show that this treatment is no longer considered “experimental” for this disease.

In addition to limited treatment options, another challenge of sickle cell disease is lack of surveillance data. However, according to a 2009 report, the Commonwealth of Virginia was home to 3,347 individuals living with sickle cell disease. Sickle cell disease patients are high utilizers of health care with frequent inpatient hospital visits, emergency department use, prescription drugs, and physician visits. According to the Centers for Disease Control and Prevention, approximately 50% to 60% of sickle cell disease patients are Medicaid beneficiaries. The promise of bone marrow transplantation for those eligible would not only allow for improved quality of life for those with sickle cell disease, but also provide the opportunity to lessen the financial burden on Medicaid.

Therefore, although BMT CTN 1503 is a prospective study, it is comparing survival and disease-related outcomes between *two treatment options that are both considered to be standard of care; neither treatment is experimental or investigational practice*. This study will yield invaluable information to guide future treatment recommendations. Bone marrow transplantation is one of few treatment options for individuals living with sickle cell disease and the only treatment option considered to be curative; consequently, coverage of this treatment is critical for this population. If you have any questions or require further clarification, please contact Leslie Brady, ASH Policy and Practice Manager at [lbrady@hematology.org](mailto:lbrady@hematology.org) or 202-292-0264.

Sincerely,



Kenneth C. Anderson, MD  
President

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<sup>1</sup> Gluckman E et al. Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation. *Blood*. 2017; 129: 1548 – 1556

<sup>2</sup> Shenoy S et al. A trial of unrelated donor marrow transplantation for children with severe sickle cell disease. *Blood*. 2016; 128: 2561 – 2567