Work group report: Advancing Hematopoietic Stem Cell Transplantation for Hemoglobinopathies

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ACBSCT meeting

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Charge to Working Group

* Identify barriers to transplantation and opportunities to more fully realize its potential for individuals with sickle cell disease and thalassemia.
* Submit for consideration and adoption by ACBSCT recommendations regarding high priority actions

Working Group Members

* Naynesh Kamani, MD
* Mark Walters, MD
* Robertson Parkman, MD
* Liana Harvath, PhD
* Bertram Lubin, MD
* Mary Eapen, MD
* Nancy DiFronzo, PhD
* Edgar Milford, ACBSCT Chair
* HRSA Staff

Working Group Meetings

* Meetings: WG has not met since the May 16 ACBSCT Council meeting
* After extensive discussion, a recommendation was sent to the Secretary regarding inclusion of the curative option of HCT for sickle cell disease in the NHLBI guidelines for management of sickle cell disease.

NHLBI guidelines

* Expert panel established by NHLBI in 2009 to update clinical guidelines for management of SCD
* Draft guidelines currently under review deal with:
  * Health maintenance
  * Acute complications
  * Chronic complications
  * Hydroxyurea therapy
  * Transfusions
* Little to no mention of HSCT as potential therapy for SCD in these guidelines

NHLBI guidelines

* The NHLBI has not yet published the final version of the 5th edition of guidelines for the management and therapy of sickle cell disease (public comment period ended 8/31/12)
* Dr. Nancy DiFronzo will provide an update on the process of how guidelines are developed and published.

Recommendation sent to HHS Secretary

* We recommend that the secretary consider appropriate mechanisms to assure that the revised NHLBI publication ‘Management and therapy of sickle cell disease’ include expert opinion about the curative option of hematopoietic cell transplantation for this disorder.

HRSA recommendation

The Council recommends that HRSA undertake educational/outreach efforts to the SCD patient and provider community to educate them about the progressive nature of SCD, increasing morbidity and mortality in early adulthood (ages 16–35 years), and the role of HSCT and its complications.

* Action Item

  HRSA is to report back to the Council about its progress on educational/outreach efforts to the SCD patient and provider community.

Discussion Topics for consideration by ACBSCT Council

* Improve access to HSCT
  * Study current status of third party coverage for HSCT as a therapeutic modality for all SCD patients
  * Remove insurance barriers where they exist
  * Continue and strongly encourage current efforts to increase minority representation in volunteer donor registries and diversify public CB bank inventory

Discussion Topics for consideration by ACBSCT Council

* Increase and sustain NIH funding to identify laboratory/genomic predictors of poor prognosis that might facilitate early referral to HCT
* Funding of clinical trials in HSCT for SCD that target gaps in knowledge that might broaden access: reduce transplant related toxicity, to evaluate risks and improve outcomes of alternative donor BMT, overcome immunological barriers to engraftment after UCBT