ADVISORY COUNCIL ON BLOOD
STEM CELL TRANSPLANTATION (ACBSCT)
U.S. Department of Health and Human Services (HHS)

Teleconference
February 4, 2011
3:00–5:00 p.m., EST

Welcome and Overview of Meeting

Edgar Milford, Jr., MD, ACBSCT Chair, called the meeting to order at 3:10 p.m. He noted that the purpose of the teleconference was to follow up on recommendations made at the November 15, 2010, meeting of the Council that must be finalized before the next Council meeting. Patricia Stroup, Executive Secretary for the Council, welcomed the members and thanked them for their participation. Ms. Stroup informed the meeting participants that following the Council members' discussion of the recommendations, the public would have an opportunity to comment. She added that the next Council will be May 11, 2011.

Dr. Milford explained that the recommendations made by the Council in November were edited by the Health Resources and Services Administration (HRSA) staff for clarity and technical accuracy, and that all the Council members received the edited recommendations for review in advance of the meeting. Additionally, he noted that once the recommendations are finalized, five will be sent directly to the HHS Secretary for consideration and three others will go directly to HRSA leadership.

Review of Recommendations to the Secretary

James Bowman, MD, Medical Director, Division of Transplantation, HRSA
Dr. Bowman reiterated that the staff's revisions were editorial in nature, and none of the changes affected the substance of the recommendations. He read the most recent version of each recommendation.

Recommendation 10

ACBSCT recommends that the Secretary recognize hematopoietic transplantation for generally accepted indications as a covered benefit for all Federal programs for which the Secretary has appropriate responsibility and oversight. This includes autologous and allogeneic blood, marrow, and cord blood transplantation. ACBSCT recommends hematopoietic transplantation be included as a required covered service for all federally-funded programs under the Secretary's purview, to the fullest extent allowed by law and that it be included as an "Essential Health Benefit" under provisions of the Patient Protection and Affordable Care Act.

Rationale: This recommendation is based on compelling evidence that autologous and allogeneic blood, marrow, and cord blood transplantation are effective treatments for a variety of life-threatening hematologic, immune, metabolic, and malignant diseases.

Discussion
The Council voted unanimously to approve the recommendation as written.

Recommendation 11

ACBSCT recommends to the Secretary that Medicare reimburse for the acquisition of blood, marrow, and cord blood products for hematopoietic transplantation on a cost basis similar to how reimbursement is made for graft acquisition in solid organ transplantation.

Rationale: The current Medicare payment structure seriously under-reimburses the cost of performing hematopoietic transplantation. The cost of graft acquisition is bundled into the overall reimbursement under the current Medicare prospective payment systems for inpatient and outpatient hospital services. This is fundamentally different from how reimbursement is structured for solid organ transplantation. This recommendation is made to improve the alignment of Medicare reimbursements for these costly but life-saving services.

Discussion
The Council voted unanimously to approve the recommendation as written.

Recommendation 12

For cord blood units incapable of bearing a full label, ACBSCT recommends the Secretary clarify that the expiration date can be placed on an attached label provided with the unit at time of release to a transplant center.

Discussion
The Council voted unanimously to approve the recommendation as written.

Recommendation 13

ACBSCT recommends the Secretary work with FDA [U.S. Food and Drug Administration] to review requirements for licensure in light of concerns over the potential for it to result in increased cost and decreased availability of public cord blood units with the goal that FDA urgently meet with the cohort of applicant cord blood banks to share and resolve specific concerns regarding licensure.

Rationale: ACBSCT is very concerned that FDA requirements for cord blood bank licensure may prohibitively increase the cost of and decrease the availability of public cord blood units for transplantation without necessarily increasing the safety, stability, potency, or purity of the units.

Discussion

Council members asked that the word "it" in the first sentence be changed to "licensure requirements" to ensure clarity. Jeffrey McCullough, MD, said that some of the issues around new licensure requirements affect transplant centers as well as cord blood banks. He asked that the recommendation be revised to suggest that the FDA meet with "the cohort of applicant blood banks and representatives of transplant centers."

The Council voted unanimously to approve the recommendation with the suggested changes.

Recommendation 14

ACBSCT recommends to the Secretary that models for remote collection of cord blood units be allowed with only limited, scientifically justified safety precautions. The Council also recommends that the Secretary allow for cord blood unit collection from routine deliveries without temperature or humidity monitoring of delivery rooms in hospitals approved by the appropriate bodies.

Discussion
Council members suggested changing the phrase "appropriate bodies" to "appropriate bodies for hospital accreditation."

The Council voted unanimously to approve the recommendation with the suggested change.

Ms. Stroup noted that the final recommendations would be sent to the Secretary within a few days. Meeting summaries and Council recommendations are posted online at http://bloodcell.transplant.hrsa.gov/

Review of Recommendations to HRSA
Robert Baitty, Director, Blood Stem Cell Transplantation Program, Division of Transplantation, HRSA
Mr. Baitty said the recommendations to HRSA were separated from the others to avoid diluting the impact of the recommendations to the Secretary. However, in the past, such recommendations have been included in a package of information that accompanies the recommendations to the Secretary. In addition, HRSA has the authority to act on the following three recommendations:

Recommendation 1

ACBSCT recommends HRSA, in providing guidance to banks regarding self-sufficiency, define financial self-sufficiency in a way that incorporates continued, rapid progress toward building the cord blood inventory urgently needed by patients.

Rationale: ACBSCT notes that the reauthorization for the C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory includes a requirement that cord blood banks receiving National Cord Blood Inventory funding provide a plan for, and demonstrate measurable progress toward, financial self-sufficiency. The Council

recognizes the importance of providing Federal funds to individual banks only as long as truly needed to achieve the statutory goal of improving patient access to transplant and transplant outcomes by rapidly increasing the inventory of high-quality, genetically diverse cord blood units. The Council also recognizes that as the use of cord blood for transplantation (and potentially for other therapies) increases, it should become possible for banks to finance appropriately high levels of collections from diverse populations through the sale of cord blood units.

Discussion
Council members suggested changing the phrase "in providing guidance to banks" to "in providing guidance to cord blood banks."

The Council voted unanimously to approve the recommendation with the suggested changes.

Recommendation 2

ACBSCT recommends to HRSA that a financial analysis of public cord blood banking be integrated with a demand (need) analysis to determine the cost of providing access to cord blood transplantation for the United States population. The Council recognizes that additional HRSA resources will be required to complete the work outlined below, but the work is essential to develop further recommendations on prioritization of HRSA resources to assure access to a suitable cell source in the future.

To complete the assessment, the work group recommends the following as next steps:

1. Complete financial analysis
    a. Analyze historical use by total nucleated cell count (TNC) for all broad ethnic groups
    b. Compare human leukocyte antigen (HLA) match rate by TNC by broad ethnic groups of donors and recipients
2. Conduct a retrospective multi-variate analysis of adult cord blood transplantation outcomes
    a. Goal is to study cord blood characteristics against recipient outcomes including survival, disease free survival, and Graft versus Host Disease
    b. Minimum variables will be pre-freeze TNC and CD34, where available; HLA match rates; colony forming units; post-thaw viability; infused TNC/kg; CD34/kg; and colony forming unit/kg cell dose and ethnicity. Other product characteristics may also be considered
    c. Include data from the Center for International Blood and Marrow Transplant Research, the New York Blood Center, the European Group for Blood & Marrow Transplantation (if workable), and individual cord blood banks, as needed
3. Integrate cord blood inventory size analysis with the financial model to assess:
    a. Cost of meeting access thresholds by ethnicity
    b. Impact on self-sufficiency scenarios

Discussion
The Council voted unanimously to approve the recommendation as written.

Recommendation 3

ACBSCT recommends HRSA's reimbursement requirements be changed to allow initiation of cryopreservation (rather than current "completion" of cryopreservation) up to 48-hours from time of cord blood unit collection. In general, cord blood units should be processed and cryopreserved as close to the time of collection as possible.

Discussion
The Council voted unanimously to approve the recommendation as written.

Interim Report to Congress
Robert Baitty, Director, Blood Stem Cell Transplantation Program, Division of Transplantation, HRSA
Ms. Stroup reminded the Council that the Stem Cell Therapeutic and Research Reauthorization Act of 2010 requires that, within 180 days, in consultation with the Council, HHS must provide an Interim Report to Congress describing 1) methods used to distribute National Cord Blood Inventory (NCBI) funds to banks, 2) how cord blood banks contract with collection sites, and 3) recommendations for improvements in NCBI funding methods to encourage efficient collection of high-quality, genetically diverse units. Mr. Baitty suggested that the section on methods of distributing funds should be a brief description of the current process. The section on how banks contract with collection sites requires some more detail about the contract process.

Mr. Baitty explained that the contracting structure is designed to allow the inventory to obtain materials rapidly, to ensure sufficient supplies are available to serve various ethnicities, and to allow for geographic dispersion in case of disaster. Funding proposals are evaluated by a technical review panel that considers technical merit, the bank's ability to assist the NCBI, its ability to quickly begin collecting and banking cord blood, past performance, discount prices, and prospects for financial self-sufficiency. Awards are made on the basis of a competitive process; funds are obligated to the banks and paid through a monthly reimbursement system. Reimbursement is determined by unit prices that are negotiated in advance and based on demographic targets and total funding available. Reimbursement is subject to availability of funds and the bank's performance. In recent years, NCBI contracts have provided incentives to increase collection of minority cord blood units by paying a higher rate for them.

For the third section of the report to Congress, HRSA will describe the pros and cons of the following options for improving NCBI funding methods to encourage collection of more diverse units:

1. Provide a small amount of upfront funding to defray the startup costs of initiating collection at new sites.
2. Eliminate the differential reimbursement for minority cord blood units.

3. Increase the differential reimbursement for minority cord blood units, possibly reimbursing only for minority cord blood units.
4. Reduce the emphasis on discounted prices.
5. Reimburse for cord blood units collected remotely from banks that don't have a written agreement.
6. Raise the total nucleated cell count (TNC) threshold for reimbursement in view of cord blood banks' strong tendency to collect the largest cord blood units.

Mr. Baitty noted that the Council is involved via work groups in examining these options and providing HRSA with expert advice. In addition, HRSA is talking with banks and the Cord Blood Coordinating Center (CBCC) for the C.W. Bill Young Cell Transplantaton Program (Program) about these options. The assessment of these options will not be completed in time for inclusion in the Interim Report to Congress due in April. Therefore, the report will not identify a specific option that HRSA intends to follow. Mr. Baitty asked Council members whether other options should be included in the report.

Discussion
Joanne Kurtzberg, MD, suggested that HRSA identify policy changes that would incorporate hospitals into the infrastructure for cord blood collections and give hospitals an incentive to participate in collections. Dr. Milford emphasized that the report is intended to describe what HRSA may do; it is not an invitation to advise Congress. Mr. Baitty also clarified that background information about cord blood collection would be included in the introduction to the report.

Robert Hartzman, MD, said the report offered the opportunity to propose a radically different approach to subsidizing the cost of banking. For example, instead of a competition to fund individual banks, NCBI might pay for individual cord blood units (e.g., those with high cell counts) and allow banks that meet certain quality criteria to participate in collection. Mr. Baitty said the law requires that NCBI write contracts and set a maximum for each contract, but HRSA could pay differentially based on cell counts. Mary C. Hennessey, JD, suggested incorporating both Dr. Hartzman's and Dr. Kurtzberg's ideas into the report by proposing to evaluate a broad range of contractual and financial models to increase the supply of high-quality units. Mr. Baitty noted that the Program's CBCC could potentially fund new collection sites instead of NCBI, but he did not believe that NCBI had the authority fund collection sites directly. Mark McGinnis of the Office of the General Counsel noted that, for the Interim Report to Congress, HRSA was specifically directed to address ways to improve the distribution of Federal funds to cord blood banks; Mr. Baitty added that the law requires that funds be used for contracts with cord blood banks.

Pablo Rubinstein, MD, cautioned that innovative models to encourage cord blood donation must adhere to the new regulatory requirements that the FDA plans to implement. He said the FDA's requirements should be considered in the context of suggestions to change funding policy and criteria. Mr. Baitty responded that the FDA requirements likely will be addressed in the description of the pros and cons of each option.

The Council voted unanimously to approve the direction proposed by Mr. Baitty for the Interim Report to Congress.

Dr. Milford encouraged Council members to contact HRSA directly with additional suggestions for the report. Ms. Stroup noted that the report is due to Congress in April.

Public Comment
No comments were offered.

Conclusion and Adjournment
Dr. Milford adjourned the meeting at approximately 4:00 p.m.

ATTACHMENTS
- Summary of recommendations

ADVISORY COUNCIL ON BLOOD
STEM CELL TRANSPLANTATION

Summary of Revised Recommendations
February 4, 2011

Advisory Council on Blood Stem Cell Transplantation Recommendations to the Secretary

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