Advisory Council on
Blood Stem Cell Transplantation (ACBSCT)
US Department of Health and Human Services
5600 Fishers Lane
Rockville, MD 20852

Thursday, March 3, 2016

Welcome and Opening Remarks
Jeffrey McCullough, MD, Chair, ACBSCT

Dr. McCullough called the meeting to order, and welcomed all council members and other participants to the meeting. He expressed appreciation for all of the members of the public on the call.

Program Report: Division of Transplantation
CAPT Melissa Greenwald, MD, Acting Director, Division of Transplantation, Healthcare Systems Bureau, Health Resources and Services Administration, US Department of Health and Human Services

CAPT Greenwald spoke about the legislative authority under the Stem Cell Therapeutic and Research Reauthorization Act of 2015. HRSA's work is authorized by that legislation, which reauthorized the C.W. Bill Young Cell Transplantation Program. The program aims to increase the number of participants in the marrow registry; to operate patient and donor advocacy services; to provide education on transplantation; and to analyze and report transplant outcomes data.

The National Cord Blood Inventory goals are to fund cord blood banks to increase the availability of cord blood. They want to add at least 150,000 units of cord blood to the national inventory, and to make additional units available for research that may not be appropriate for transplantation.

The reauthorization requires HRSA to report on the state of the science for using adult cells and birthing tissue to develop new therapies and for inclusion in this program.

Another new piece of legislative authority is that, by December, HHS is to issue determinations with respect to inclusion of peripheral blood stem cells (PBSC) and umbilical cord blood in the definition of human organs. The determination will affect whether PBSC or umbilical cord blood donors can be reimbursed. This is prohibited with bone marrow for bone marrow donors.

CAPT Greenwald also reviewed the budgetary amounts appropriated for Fiscal Year (FY) 14 and FY15, and projected amounts for FY16 and FY17.

The program serves a growing number of patients in need of unrelated donor transplantation. As of September 30, 2015, the registry included 13.6 million adult donors. More than 3.4 million (approximately 25 percent are self-identified as belonging to a specific racial or ethnic minority.

In FY15, the program facilitated 6,373 transplants, a 1.9 percent increase over FY14. That fiscal year, there were 5,078 transplants in the US, a 3.8 percent increase from the previous fiscal year. Thus, cord blood is still being used. The total number of Cord Blood Units (CBUs) shipped last year was just under 1400.

For funding priorities, HRSA may consider funding cord blood banks (CBB):

- Collecting only, or a high percentage, of minority (CBUs);
- With proven experience in meeting established goals;
- That have received FDA licensure;
- That offer the government significant discounts or best value;
- That are financially stable; and
- That are able to expand CBU collections and deliver more CBUs annually.

HRSA is also identifying special projects that will increase the diversity of the National Cord Blood Inventory (NCBI), as this is a priority.

HRSA's Division of Transplantation, Blood Stem Cell Transplantation Branch, assesses data regarding the number of unrelated blood stem cell transplants facilitated through the CW Bill Young Cell Transplantation Program. This is done, partly, to better understand the role of haplo-identical transplants and other therapies, including their impact on cord blood utilization and the impact on under-served populations. They are also hiring more people to examine available data.

CAPT Greenwald said that they would like to have future conversations on a wide variety of topics concerning the science and new findings.

Discussion

Dr Mary Laughlin said that it is important to examine trends, because the field is moving from the team use of double corporate transplants to single ones. They should also be looking at the use of various graft sources. She recommended not limiting the use of literatures that is claiming efficacy based on certain graft sources.

Another participant agreed on the direction in which the field is moving.

Dr. McCullough noted that the program has been given an exciting, broad direction from the authorizing legislation. *CAPT Greenwald* agreed that the changes in legislative authority certainly do move them in new directions. It is

clear that they have the ability to make decisions on payment issues. The 2019 report looking at the field of cell transplantation in general and how the program might be expanded will require a lot of thought and partnering with stakeholders, including those on this advisory council. They need to think about the changes that have been happening in the scientific field and the direction they want to take now.

Dr. Sergio Giralt asked if other kinds of therapy will come under the program's review. *CAPT Greenwald* thinks that they will be looking at various ways HRSA can be involved in those areas.

Dr. E.J. Shpall said that they have been using cord blood for a number of purposes at MD Anderson in Houston. It seems like HRSA is very supportive of exploring non-transplant strategies. An unnamed participant said that this was included in the original legislation for the program. Dr. Shpall said that they have just opened a study at Duke using non-NCBI cord blood units for a stroke trial.

Recommendations of ACBSCT Work Group for Improving the Availability of High TNC Cord Blood Units for a Diverse Population

- *Karen Ballen, MD, work group co-chair*
- *Mary Hennessy, JD, work group co-chair*

This work group was challenged to make recommendations to adjust the funding and reimbursement systems to support continued collection of CBUs from minority donors.

Ms. Hennessy recognized the participation of the entire work group.

The work group realized that the point is to ensure that the national cord blood inventory continues to serve a diverse population. They have to ensure that CBBs are able to participate in the inventory and that they are appropriately reimbursed for collecting and banking units from diverse backgrounds.

The group found that cord blood units with higher Total Nucleated Cell (TNC) counts are being used more than units with lower TNC counts. This reflects a strong clinical preference for units with high TNC counts.

Of the transplants performed, a higher percentage of pediatric patients use small CBUs than the percentage of adult patients, but there are more transplants for adults than for pediatric patients.

Transplant centers use various combinations of match requirements and TNC dose. There are no universally accepted match requirements. Patients using smaller units tend to be younger, and very few patients get a 6 out of 6 Human Leukocyte Antigen (HLA) match.

Minority patients tend not to find as close matches as Caucasian patients. African American patients are more likely to find a suitable match from African American donors, which typically have lower TNC levels due to biological factors. As a result, minority patients receive smaller units to a greater extent than Caucasians, and more low TNC units have been banked for minorities than for Caucasians.

Generally speaking, adult cord blood use in the U.S. is static or declining, while pediatric and non-malignant cord blood use is increasing. This is consistent with what is happening in Europe.

The data clearly shows that HRSA funding is essential to ensuring that there is an adequate cord blood supply to serve the public. Thus, HRSA funding may influence decisions about what size units the CBBs collect and bank. HRSA funding also influences the diversity of the registry.

The NCBI has more minority CBUs than non-NCBI banks, reflecting HRSA's important role. It has more lower TNC units than higher TNC units. Additions to the inventory will continue to incrementally improve matching options. Patients would now have the opportunity for greater selection, due to the growth in the inventory. Some banks have already shifted to a higher TNC cutoff on a voluntary basis. European banks are also shifting to a higher TNC cutoff for cost effectiveness reasons and to match clinical interest in the higher TNC levels.

Dr. Ballen said that the National Marrow Donor Program (NMDP) conducted a survey, and found that most banks support a shift to a higher TNC cut-off. However, this would require additional compensation because many more units would need to be collected in order to bank the higher TNC level units. Transplant physicians also support the shift to an inventory of larger CBUs.

She also went over unknown information, such as the impact of haplo-identical transplants on the demand for CBUs. Other technologies are also expanding that could impact the demand for CBUs, as well as alternative therapies, such as regenerative medicine.

The work group made the following recommendation:

> HRSA should adopt a funding framework that incentivizes the collection of high TNC units for a diverse population that recognizes the higher associated costs
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> - Greater incentive to add units to the inventory that have higher TNC, including for minorities, which are associated with better outcomes
> - Recognizes growth and diversity of inventory since its inception and creates incentive to shift banking toward most-needed units

- Recognizes that per unit reimbursement will need to reflect higher cost per unit banked for higher TNC, minority units
- Need to expand collection activity
- Fewer total units will be banked

Dr. Ballen and Mike Boo presented two examples of how this recommendation could be implemented, including the financial aspects.

Implications of the first example, would be a higher cell count threshold, which would require collections to be expanded. The net effect would be a smaller number of total units banked, but a larger number of high TNC units banked.

The second example resulted in feedback from CBBs that they would need more time to ramp up to the higher TNC requirement. Under this example, banks would receive higher reimbursement as TNC levels increased per unit. The net gain in large units would be smaller in this case, because smaller units would continue to be reimbursed to a limited extent.

The second example could be easier to implement for some CBBs, especially those serving minority groups. It would also be more complicated administratively.

The work group recommended amending existing contracts within the next year, rather than re-bidding them. They suggested increasing the per unit funding for higher TNC units and for diversity. HRSA contracts would need to be individualized to fit the accrual targets and patterns of each CB bank, with different race and ethnicity requirements.

HRSA and CBBs should commit to pricing that incentivizes the collection and banking of high TNC CBUs for a diverse population, and that provides for higher reimbursement. The emphasis should continue to be upon ensuring that the banks fully meet the needs of minorities, especially African Americans.

The work group envisions one year needed to amend the contracts, with an outcome review at the18-month and three-year marks.

They also suggested that HRSA should fund demonstration projects for CBBs using innovative ways of collecting and banking the desired CBUs. All CBBs should be encouraged to participate in these demonstration projects. Since some report having difficulty in meeting these projects' goals on their own, the work group suggests that the CBBs should be encouraged to partner with each other to accomplish larger projects.

Discussion

Upon request, HRSA staff explained the reimbursement system currently in use. CBBs submit their expected costs, and HRSA signs a five year contract to cover those costs with some discounts. There is no expectation that HRSA will purchase every unit the bank produces. They acknowledged that the various CBBs calculate their costs differently.

Currently, any CBB can propose the recommended payment scheme if they so desire when contracting with HRSA. However, they tend to be having financial issues. Thus, they are unlikely to voluntarily give up the current reimbursement they can receive for the units with lower TNC levels.

A participant said that they must be certain that increased costs are not being passed onto the transplant centers.

HRSA will need to determine whether, under the new policy, what is happening to units that are not eligible for transplant purposes. They could be used for research instead, for example.

A participant said that the Council should continue to look at what additional incentives may be needed for the collection of minority units at the higher TNC level. They should also look at the impact of additional incentives and finances for the collection of Caucasian units.

The work group's financial examples are based solely on estimates of what the CBBs receive from HRSA for collecting the CBUs. Each blood bank has a separate, confidential contract with the government.

Currently, one of the reasons transplant centers have to pay so much for each unit of blood is because so much of the inventory is taken up by smaller units, which cannot be used for transplantation. The work group thinks this cost may actually go down under the new framework, since more of the inventory will be made up of units that can be used for transplantation.

The recommendation itself is an overall one, and is tied to ultimately encouraging HRSA and the CBBs to agree to pricing that incentivizes the collection and banking of high TNC units.

The recommendation has been discussed with the medical directors of the corporate blood banks. The work group asked them to discuss it with their financial and other representatives at their CBBs. The banks have indicated that they support the higher TNC, as long as the financing recognizes the need for more collection of units overall.

The recommendation is purposely made non-specific enough to give HRSA flexibility and room in which to work with each blood bank to suit their financial

needs. HRSA representatives agreed that this is “very doable”, and actually reflects what the agency is already doing.

Several participants called for moving ahead on this recommendation. Waiting will only give things time to get worse financially for the blood banks.

The recommendation was adopted unanimously.

Financial Health of Cord Blood Banks
Merry Duffy

The economics of this sector is “very bad”. This Council and HRSA have an obligation to work to keep them financially viable.

Ms. Duffy said that 90% of CBBs worldwide are struggling to maintain sustainability and avoid bankruptcy. The largest source of revenue (only source for many) is the sale of CBUs for transplantation, which covers 81% of the industry’s operating costs. CBBs will need to process and bank units based on HLA (diverse) and TNC (large) to meet selection criteria. The challenge is not only to provide compatible units of sufficient size for every patient, but to be able to afford to do so.

Several factors have worsened the situation for banks in recent years. CBU usage is declining worldwide. Licensure requirements have increased the cost of banking blood. Banks have attempted to move into collection hospitals with high numbers of minority donors. However, this is a lengthy, expensive process. Remote collection programs also faced expensive challenges. Developing partnerships with for-profit entities has not been easy, with mixed success for the CBBs. Finally, transplant center anxiety over the cost of cord blood transplantation has increased.

In the past few years, two banks have closed and another two have stopped or will stop collecting completely, but will still distribute inventory. Four banks have significantly reduced the number of their collection hospitals. One bank that spent several years preparing to open has abandoned the idea, and is giving away its materials. These consequences have happened due to financial issues, including low utilization and concerns over obtaining licensure.

In addition, people at HRSA have noticed that the CBBs appear to be experiencing high rates of staff turnover.

Thirteen U.S. CBBs responded to a survey about their financial situation. Responses from two of the banks were not usable. Of the remaining 11, none were profitable in 2015. Nine banks reported no profitable years out of the last five.

Three banks reported that they did not recover direct operating costs in the last five years. Two banks covered their costs with other revenue; one bank covered costs with revenue from the "good years" (more than five years ago); two banks recovered their direct costs in only three of the five years and one bank recovered its direct costs in only one of the five years. One bank has managed to break even, but never managed to recover the costs of obtaining licensure.

All 13 banks provide units for research, and all but one charge at least a minimal fee. To charge fees and how much to charge is based on the purchaser. Fees are not a revenue source.

The survey asked the CBBs what they intend to do to address their financial problems. The banks replied with a number of strategies: raise the minimum TNC; reduce or discontinue collections; increase the price; and outreach to promote cord blood. The CBBs called for some resolution on the licensure question, with either all banks being required to meet those standards or none. They are also looking to improve efficiency, through enhanced collector training and assessments of the collection to banking ratio. It was suggested that the efficiency area could be a good target for demonstration projects.

The cost of obtaining licensure is steep, and can reach into the millions of dollars. So far, only six banks have been licensed.

Many CBBs were also considering doing business in other product lines, such as tissue procurement and consulting agreements. Several CBBs were also looking at branching into the private sector, since public banks do not appear to be profitable on their own.

Discussion

The survey did not ask exactly how much the CBBs are losing, in terms of dollar amounts.

The work group agreed upon the following statement:

> The Council recognizes the serious financial challenges confronting the cord blood bank sector. In order to assure sustainable cord blood bank collection and supply for the future, the Council requests that HRSA engage experts to conduct a study of the economics of the U.S. cord blood banking system with the intent to identify current business practices and make recommendations for ways to strengthen the financial operation of this sector to assure long term function.

The participants, in general, supported getting more information on what is actually happening on the financial side of the sector. There was concern, however, in having consultants who usually focus upon the corporate world look at this private sector market.

The high costs of licensure have been the “real killer” for the CBBs. More should be known about how much of the financial problems are related directly to the actual collection and banking of blood, and how much to the additional regulation.

The suggestion was made to change the last part of the paragraph to “assure long term sustainability”, rather than function.

The Recommendation was adopted by the Council on a unanimous vote.

NIH: BMT Late Effects Initiative

- *Minoo Battiwalla, Intramural NHLBI/HB*
- *Shahrukh Hashmi, Mayo Clinic*
- *Navneet Majhail, Cleveland Clinic & ASBMT*
- *Steve Pavletic, Intramural NCI/ETIB*
- *Bipin Savani, Vanderbilt University*
- *Noniekaye Shelburne, NCI/DCCPS*

Dr. Battiwalla said that transplantation numbers are increasing worldwide. The relapse risk continues to decline. As a result, there are a growing number of survivors with health concerns, of all kinds.

Short-term survivability of transplant patients has improved significantly. However, they still face higher mortality rates in the long term. Researchers still do not have a good handle on their causes of death in many cases.

In the first two to three years, the main problems are relapse and infection. In the long term, there are increasing levels of endocrine and eye problems, and also cardiovascular limits showing up. There are hints that these people are facing premature aging.

Some of the long-term complications are potentially lethal. Currently, this information is based only on observational studies, and the pathobiology is poorly understood. Researchers do not really know the best effective method of screening and preventing these complications.

NIH has selected a set of experts around the world to examine these issues. They set up working groups in six critical areas unique to transplantation: healthcare delivery; research methodology and study design; new malignancy; quality of life and psychosocial outcomes; immune dysregulation and pathobiology; and vascular and metabolic issues.

The working groups have been encouraged to focus upon the most challenging issues in that field. Some things may be high frequency but of minor concern, as opposed to rarely occurring events that are “biologically interesting”. They want

to focus upon the future (10-20 years from now), to see what is coming down the road.

The groups are also encouraged to involve specialists from other, related fields.

Understanding these issues among the transplant population could lead to information useful for other populations that suffer from these conditions.

The first planning meeting took place last June. Since then, they have been holding working committee teleconferences. The working committees presented their draft presentations last month. The final NIH Consensus Conference is scheduled for June 21 and 22 at the Shady Grove campus. They expect to have a lot of federal partners at that conference. They hope to stimulate research prioritization and program announcements.

In the long run, this information may influence how they collect data collection for the common registry that might affect transplant outcome assessments.

Discussion

HRSA has provided a little bit of seed funding for the initiative's first year. Dr. Battiwalla hopes to be able to continue to grow that funding throughout the federal system.

Dr DiFronzo said that this plan will be used for funding prioritization, and they are already looking at the information in that way.

Cellular Therapy Registry
Marcelo C. Pasquini, MD, MS

Dr. Pasquini explained that the registry was started to collect information on patients receiving cell therapy for purposes other than transplantation. Over time, the collection of data will allow centers to retroactively send information whether they performed cellular therapy over a period of time starting in 2002.

They captured infusions done in 606 patients. Most were for neurologic disorders. They noticed that, in the last two years, some centers started reporting for acute leukemia and other disorders. They received 30 reports of patients receiving CAR-T cell therapies.

It is now much easier to categorize and classify transplantation based on stem cell sources.

The reporting process is still very complicated. The group intends to continue to have relationships with the blood banks, but also with the sponsors and the regulatory agencies in order to collect more data.

The task force looked at how to build upon the exiting infrastructure to develop a cellular therapy registry for research purposes. They want to develop a cost effective tool for long-term follow up for cell therapy trials, and to increase center participation in this initiative.

The registry changed the form to Cellular Therapy Essential Data Form (CTED). The pre-CTED is triggered whenever a cellular therapy is done. A follow up structure will be established for submission of post-CTED appropriated to each cellular therapy indication. They will be creating CRF forms for certain indications, develop an infrastructure for collecting all of the information.

All existing indications will be retained in the registry. However, certain indications will be prioritized, since they are of higher interest to those who will be using the registry. They will also prioritize certain products, such as genetically modified cells and multi-virus-specific T-cells for infection. The Food and Drug Administration (FDA) already requires collection of 15 years of information on patients who receive the genetically modified cells. The idea is to centralize any cell therapy that is not performed as part of a transplant.

Information on both cellular therapy and hematopoietic cell transplantation will be collected in the registry.

The pre-CTED will collect demographic and disease information. The infusion form will collect information on the product, including manufacturing and product analysis, as well as infusion details. The post-CTED form will collect information on follow-up infusions, recipient survival and disease status; cause of death; development of secondary or additional malignancies; persistence of the product; and development of Cytokine release syndrome (CRS).

If the patient receives several infusions, a separate infusion form will be filled out for each.

Comprehensive reporting will occur for cellular therapies, with additional information from that on the CTED.

It becomes very difficult to break down the product based on manufacturers information. Thus, the registry will collect additional information on the source and type of information about the product in a number of areas. They expect that some companies will already have this information laid out, so that this information will all be known based on the product's name and id number. The goal is to characterize products in a way that allows comparison. They are working to figure out how to help data managers in situations where the information is not readily available.

There are several cellular therapy scenarios that must be accounted for. They need to figure out how to structure the information collection in such a way as not to overwhelm those entering it into the registry.

Centers will receive reimbursement for collecting the data similar to Hematopoietic Stem Cell Transplantation (HSCT). Pilot data collection will take place in centers already actively doing these cell therapies. The cell processing laboratories will also be included. In addition, one institution could have several programs that contribute data to the registry.

For long-term follow up on genetically modified cells such as CAR T cells, centers want to have an ability to capture all of the parameters that apply to the transplantation and to care quality. This is not all that different from what has been done with other registries. A member expressed concern that companies could develop their own registries to comply with FDA requirements to perform long-term follow-up. Creating a central registry would hopefully prevent that, and collect the information in a form more usable for researchers.

A lot of the funding so far has come from non-governmental sources.

The CTED forms are completed. They will probably be launched in the summer of 2016. They are currently adapting disease forms to also collect data on cellular therapy. The changes on those forms will probably be minor.

European Group for Blood and Marrow Transplantation (EBMT) is working in a parallel fashion with CIBMTR. The two groups are discussing the best way to reach harmonization.

Discussion

A participant noted that the cost of reporting is becoming very burdensome for users. They have to make the system much more efficient. One participant expressed a concern that research centers will give up that role out of frustration. Another one said that she hopes a lot of this reporting will, someday soon, become very automated.

Private commercial vendors do not see the value in providing these efficiencies in data reporting. A participant called for a central organization (such as the government) to make the system more efficient and easier to participate in.

The FDA, not HRSA, is going to require the companies to maintain long-term follow-up of individuals who receive cellular therapies.

HRSA Requirements for Cord Blood Bank Accreditation
Anita Wabeke, Lead Public Health Analyst, Division of Transplantation, Healthcare Systems Bureau, HRSA

Ms. Wabeke said that HRSA is looking for the Council's input on this accreditation issue. The issue is that, when an NCBI CBB is accredited by more than one entity and its accreditation is suspended or terminated by one, whether HRSA should be taking any action.

Legislatively, the Secretary is to recognize at least one accreditation entity for CBBs. The Secretary is also to ensure that informed consent is obtained by the CBBs.

Ms. Wabeke quickly summarized the actions taken to date by HRSA on accreditation.

Discussion

This situation has happened, although the Council is not asking to consider any particular instance.

CBBs are expected to notify accrediting bodies if something happens to their approval from another accrediting body. The second accrediting organization then has the choice of whether to continue to accredit the CBB or require additional information or oversight.

Zika Virus Update
Matt Kuehnert, MD, Director of the Blood, Organ and Other Tissue Safety, Centers for Disease Control and Prevention (CDC)

Dr. Kuehnert provided an update on the Zika virus, transmitted by the Aedes mosquitoes.

He said that only about 18% of adults infected report any symptoms. More than half in one study reported a rash, fever, arthralgia and conjunctivitis. He said that "it looks like dengue", in that it has similar clinical features. The same kind of mosquito carries both viruses.

The clinical illness is usually mild, and symptoms last several days to a week. It is uncommon for hospitalization to be required. However, Guillain-Barre syndrome has been reported in patients following infection, particularly in the current outbreak in Brazil.

Microcephaly among infants is now being linked to the Zika virus. Most of the data on this has come from Brazil. From a pathology standpoint, there is high evidence of the virus causing this syndrome, as well as early fetal loss and neonatal death.

There are a number of diagnostic tests available from public health laboratories. The different tests use varying samples at different times after infection. These are public health tools, and will not be widely available for screening.

There is no specific antiviral therapy. Recommended treatment is support for whatever symptoms occur. However, aspirin and other Nonsteroidal anti-inflammatory drugs should be avoided.

There is no vaccine or medication to prevent infection. Pregnant women should avoid travel to affected areas, and others should take steps to avoid being bitten by mosquitoes.

There is a high probability that Zika can be transmitted through blood transfusions. There are also reports of sexual transmission, and the virus appears to live for a long time in male sexual reproductive tissues.

There are no FDA-approved tests for screening donors of blood or tissue. Screening would most likely first occur under an investigational new drug protocol.

Guidance has been put out by FDA for blood donor screening. Donors with a recent history of travel to affected areas or sexual contact with a traveler should be deferred for at least four weeks.

FDA has also produced recommendations to prevent tissue transmitted Zika virus infection, for things such as human cells, tissues, and cellular and tissue-based products. They are divided into deceased and living donors, and how to handle those products. The FDA is still soliciting input on the guidance, but hopes to finalize it soon.

Discussion

There was brief discussion on the reasons behind having different requirements for living and dead donors.

HRSA Funding of Transplants

The Council considered the following recommendation:

> The ACBSCT recommends that the Secretary encourage the Centers for Medicare and Medicaid Services (CMS) to reimburse for hematopoietic stem cell transplant regardless of if the transplant is performed as an inpatient or an outpatient, provided appropriate documentation of the transplant procedure exists.

A Council member said that, under Medicare, reimbursement is “horrifically low”. In addition, there have been incidents of audits pointing to this as an area in which money can be recovered by the Medicare program, and hospitals having to pay money back.

Currently, CMS does not reimburse for transplants performed on outpatients. In that setting, payment would be approximately $1000. If the patient were to be admitted to the hospital for the procedure, and stays for 48 hours, the cost rises to approximately $26,000.

Shelley Grant said that this concern could be informally raised with colleagues at CMS. It could also be included in an interagency work group. Dr. McCullough said that would be great, and the issue will be brought up again at the meeting in the fall.

NCBI Eligibility and Qualifications: disparities between banks with a Biologics License Application (BLA) and those continuing to bank under IND

Joanne Kurtzberg, MD

Dr. Kurtzberg said that, after finalizing the guidance for BLA public CBBs, HRSA modified the contracts of banks receiving their BLAs to limit accrual to licensed units. Thus, eligibility for the NCBI differs for CBUs banked in licensed or unlicensed banks. There are six banks with a BLA, and 11 without.

This creates additional hardship for the licensed banks. Units that would qualify for distribution under the National Marrow Donor Program (NMDP) INDs are not reimbursed by HRSA if banked by a licensed bank, but reimbursed if the bank does not have licensure. Thus, licensed banks may forgo banking valuable units of blood. HRSA has not set forth any timeline by which CBBs must be licensed.

In addition, licensed banks have had otherwise IND qualified units removed from funding, and have been required by HRSA to replace those units. These are the same units that would be acceptable to HRSA if the bank was unlicensed.

She presented several examples of units that were suddenly not eligible once the bank was licensed.

Other than the BLA change, NCBI eligibility requirements have not been updated for 10 years. There have been changes in how CBBs operate today, and these requirements should be updated. All accruals to the NCBI should be based on these requirements, rather than the licensure status of the CBB.

Dr. Kurtzberg made the following recommendations:

- Accrual to the NCBI should be based on NCBI requirements that apply to all banks.

- The licensure status of the bank should not be included in NCBI eligibility.
- HRSA requests for replacement of IND units meeting NCBI eligibility and banked by licensed banks should be cancelled.
- The NCBI eligibility requirements should be reviewed and updated by members of the Cord Blood Advisory Group and Cord Blood Coordinating Center, and possibly others.

Discussion

Dr Tom Price asked if there are a lot of units involved. Dr. Kurtzberg said that she is aware of hundreds of units being so involved.

Dr. McCullough asked Shelley Grant if she can do anything on this issue before the Council meets again. Shelley said that most contracts stipulate that, once a CBB is licensed, HRSA will only pay for units acquired under the licensure requirements. It could be that some units started out as licensed, and then became ineligible. Other banks may have misunderstood the requirements and mistakenly billed the government. This may account for those units that the government is requiring be replaced.

Dr. McCullough suggested that the group devote a "fair amount of time" to this complex situation at its next meeting. Dr. Kurtzberg said that they need a task force to review the NCBI requirements and to make recommendations for updating it. She would like for this Council to recommend that. She said that the situation is one in which licensed banks are, quite simply, being penalized. In her institution's case, HRSA will not allow any units that were collected earlier on the day they received the license.

Shelley said that it would be appropriate for the Council to recommend the creation of a task force to consider updating the NCBI requirements. The Council Chair agreed that he, Shelley and a few other people should start considering the creation of a task force to consider these issues.

Public Comment

Mary Laughlin said that the Council should consider that the regulatory burdens have been a significant burden. They are all looking for a path to helping relieve the public CBBs of some of this burden.

Announcements

Patricia Stroup said that they are seeking nominations for the Council. Names should be forwarded to her.

The next meeting will be September 13 and 14, in Rockville, Maryland. This will be an in-person meeting.