

AMERICAN SOCIETY FOR BLOOD AND MARROW TRANSPLANTATION

**POSITION STATEMENT ON FDA GUIDANCE DOCUMENT
FOR LICENSING CORD BLOOD BANKS**

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The intent of a licensing plan for cord blood banks is laudable. Our entire membership desires safe, pure and effective cellular products for our patients requiring umbilical cord blood transplants. However the plan, as presented, could have serious unintended consequences for patients if it limits access to cord blood products collected in non-licensed facilities or collected before the establishment of licensing requirements. If licensing is needed, it should be implemented only in very carefully measured steps. Here are the reasons:

- The selection of cord blood products is the practice of medicine. The transplant physician identifies the unit with the optimal cell dose and HLA match for a patient, or the best pair of products for double cord blood unit recipients. Clinical research has shown that these factors are the most critical to the success of umbilical cord blood transplantation for the patient. To deny a patient access to the most appropriate product would compromise their care and could threaten their survival.
- In public cord blood banks around the world there are at least 250,000 units registered with Bone Marrow Donors Worldwide (BMDW) and available for unrelated donor transplantation. It is likely that many of these units have been collected or stored using protocols that do not precisely match the proposed requirements for U.S. licensure. But there also is little reason to conclude that many or most of these units are not safe or efficacious.
- Because of the diversity of the U.S. population, often the best cord blood product may be located in another country. The volume of imported units that are selected and transplanted is about 20 percent and growing. The need for imported cord blood products is large and expected to grow because of the genetic diversity of Americans. The availability of these cord blood products is especially important for racial and ethnic minorities.

- To deny access to a unit of cord blood because a facility has not met a U.S. licensing requirement could limit chances for treatment and shorten survival for a patient if that unit has been selected as best in terms of cell dose and match. In such instances, licensing requirements designed to increase safety could, instead, contribute to mortality. We support efforts to establish standards and to encourage all donor banks and processing laboratories to meet those standards, but it is essential to allow the clinician to decide on the safety and efficacy on transplantation of a particular product.
- Unlike commercially manufactured drugs that are mass produced, each cord blood product is unique and might be uniquely suited for any particular patient. Currently the transplant physician has ultimate responsibility for selecting a product that has been collected, stored, tested and transported in accordance with appropriate, recognized standards.
- The professionally recognized standards within the transplant community are those that have been developed by FACT-NetCord, which administers an international system of cord blood bank accreditation. The FACT-NetCord accreditation program is expanding rapidly in pace with the industry both in the United States and around the world. Although accreditation plays a major role in the clinician's decision to select a product for transplant, accreditation cannot be the sole deciding factor. There can be valid reasons, based on clinical judgment, to select a unit from a non-accredited bank.
- Both FACT-NetCord accreditation and the recently enacted U.S. Stem Cell Therapeutic and Research Act of 2005 (Public Law 109-129), administered by the Health Resources and Services Administration (HRSA), already require the evaluation and reporting of outcomes of therapy for all allogeneic stem cell transplants, including cord blood transplants. We fully support these requirements for the recording, analysis and retrieval of quality outcomes data. These standards and requirements will allow the transplant community and the government to assess the efficacy of units obtained from individual cord blood banks.

- The banks able to comply with the proposed licensing requirements are most likely in the United States and Western Europe – although some requisites such as testing in a CLIA-certified laboratory would be problematic even throughout most of Europe at this time. If licensure were to limit access to cord blood products, the patients at greatest disadvantage would be those whose ancestry is non-Western European. These populations already are at a disadvantage because of a smaller pool of suitable unrelated volunteer donors of bone marrow and peripheral blood.

In summary, we know of no major safety or efficacy problems with the current system in which the patient's physician is the arbiter and ultimately responsible for the selection of cord blood units. Licensure of cord blood banks should not limit access to cord blood units collected in banks without or prior to licensure because the unintended consequences could be catastrophic for many patients. Transplant physicians have reliable ways to assess quality, such as the FACT-NetCord standards and accreditation.

Some degree of international regulation of cord blood banks may be appropriate in the future. We would urge the agency to continue watchful waiting to evaluate how the therapy evolves under the current system of clinical judgment by the physician based on standards and accreditation. We urge caution in establishing licensure requirements only in carefully measured steps if and when circumstances warrant it.