CORD BLOOD ASSOCIATION

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December 14, 2020

European Commission
SANTE B.4 – Medical Product: Quality, Safety and Innovation

Re: Revision of the E.U. Legislation on Blood, Tissues and Cells

To Whom It May Concern:

The Cord Blood Association (CBA) appreciates the opportunity to submit comments regarding the revision of the Blood Directive 2002/98/EC and the Tissues and Cells Directive 2004/23/EC that have helped maintain quality and safety for millions of patients undergoing blood transfusion, transplantation and medically assisted reproduction.

Who We Are

The CBA (www.cb-association.org) is an international nonprofit organization that promotes both public and family cord blood banking, with the objective of accelerating the use of cord blood and perinatal tissues to benefit patients and advance medicine.

Our priorities are quality products and services, research and development, advocacy, and public and professional education. Members of the CBA include both public and family banks and individuals in or served by the cord blood community including cord blood bank personnel, research investigators, laboratory technicians, patients, donors, customers, and health care providers such as transplant physicians, obstetricians, pediatricians, nurses and midwives.

The CBA vigorously promotes evidence-based medicine and the advancement of health care through clinical trials. *STEM CELLS Translational Medicine* is the association's official scientific journal.

To assure high standards among our member banks, the association requires that they be accredited by either AABB or the Foundation for the Accreditation of Cellular Therapy (FACT), or in the process of accomplishing accreditation.

In addition to having member banks on six continents, the CBA has a European Section comprising many of the leading banks in the European Union.

Our Comments

The comments that we wish to provide are divided into five sections: 1) a brief background on the scope and potential for cord blood and perinatal tissue therapies, 2) the need for updated rules and regulations for cord blood and perinatal tissue banking, 3) the alignment of cord blood and

perinatal tissue-derived products with cell and tissue therapies, 4) our commitment to harmonization of regulations across governmental jurisdictions, and 5) a desire to interact with the Commission as it moves forward on an update of the blood, cell and tissue rules and regulations.

1. Brief Background on the Scope and Potential for Cord Blood and Perinatal Tissue Therapies

   Since the late 1980s, umbilical cord blood and related birthing tissues have evolved from medical waste to a rich source of blood stem cells that can be transplanted to restore normal blood and immune cell development and function in blood diseases such as leukemia and lymphoma, certain inherited genetic disorders, bone marrow failure and immune deficiency diseases. At least 80 blood cancers, genetic diseases and immune system and metabolic disorders can be treated or cured with hematopoietic stem cell transplantation (HSCT) using cord blood as the source of donor cells.

   The search for a suitable donor for patients who need a hematopoietic stem cell transplant begins within the family. Brothers and sisters with the same parents have a 25% chance of being a perfect match. However, about 70% of patients do not have a match within the family. For them, physicians can search registries around the world for a suitable adult bone marrow donor or banked stem cells derived from donated umbilical cord blood. Cord blood has the advantage of being able to cross HLA barriers and to be transplanted without full matching. This has increased access to HSCT for patients worldwide.

   Among unrelated hematopoietic stem cell transplants today, cord blood is the source for about 40% in pediatric patients and 10% of adult patients. Cord blood as a cell source for hematopoietic transplant has several advantages over adult bone marrow. Cord blood stem cells typically have not been exposed to viruses, chemicals and environmental pollutants that can alter cell function. Because of increased tolerance of cord blood T-cells, cord blood stem cells do not have to be matched as closely to the patient as do cells from adult donors. In addition, cord blood is easily banked so it is more quickly available than obtaining stem cells from an adult donor who must be located, consented, tested and harvested. As such, cord blood can be the preferred donor source for patients who have a life-threatening genetic disorder, need a transplant quickly, or have an uncommon tissue type because of their racial or ethnic heritage.

   A growing body of clinical study data suggests that cells contained in umbilical cord blood or cell therapies manufactured from cord blood or other birthing tissues may also be able to facilitate repair of injured organs, modulate inflammation to prevent tissue damage after injury, treat cardiac and vascular disorders, modify immune conditions such as diabetes, and treat neurologic disorders including autism, cerebral palsy, spinal cord injury and stroke.

2. The Need for Updated Rules and Regulations for Cord Blood and Perinatal Tissue Banking

   The current E.U. directives for human blood, cells and tissues were enacted 16 or more years ago. All facets of public and family cord blood and perinatal tissue banking – donation, collection, processing, testing, storage and selection – have advanced significantly over that

period of time. To be comprehensive, the rules and regulations should establish minimum standards for the many critical aspects of bank operations and management, including:

- Organizational structure and personnel requirements
- Safety
- Validation of critical procedures
- Proficiency testing
- Agreements with external parties
- Donor (i.e., parental) consent
- Maternal and infant donor evaluation
- Confidentiality
- Labeling and tracking
- Change, control and process
- Documents and records handling, control and storage
- Electronic record systems
- Errors, non-conformance, deviations and adverse events
- Equipment, critical supplies and reagents
- Inventory management and storage stability
- Clinical outcome data collection and management
- Quality review, internal audits and reporting
- Operational sustainability

Many of these essential aspects of cord blood and perinatal tissue banking are not adequately addressed at the level of the E.U. or its member national jurisdictions. This deficiency is hardly unique to E. U. countries.

3. Alignment of Cord Blood and Perinatal Tissue-Derived Products with Cell and Tissue Therapies

   Cord blood banking is more aligned with cell and tissue banking and therapies than with transfusion blood banking. In fact, in the United States, cord blood is regulated as a tissue, not as a blood product. Yet, too often cord blood and perinatal tissue banking is placed under rules devised for blood transfusion banking. Other than both having the words “blood” and “banking” in their name, transfusion blood banking and cord blood/perinatal tissue banking differ in their purpose, donors, collection methods, manufacturing, testing, storage and cell selection.

   For these reasons, cord blood banking needs to have its own rules and regulations, separate and apart from transfusion blood banks.

4. Our Commitment to Harmonizing the Regulation of Cord Blood Banking

   A substantial volume of the umbilical cord blood used in clinical therapies is transported between countries within the European Union, as well as shipped to and from countries around the world. The unit of cord blood that is the best biological match for a particular patient frequently has been collected, processed and stored in another country.

Unfortunately, the laws and regulations for donor screening, cord blood testing, and cord blood banking vary greatly from country to country, thereby creating uncertainty about quality and complicating the movement of critically needed cells.

For this reason, the CBA recently created model criteria for banking rules and regulations. These minimum requirements for national-level laws and regulations were developed by banking representatives of 15 countries over a period of 18 months and published last year. Their purpose is to (a) harmonize regulations among countries where regulations currently exist, and (b) guide the development of regulations in countries that have few regulations.

Our association's development of the model criteria for regulating cord blood banks and cord blood banking is evidence of our commitment to high quality, safety and harmony in the regulation of cord blood and perinatal tissue banking. We would welcome the opportunity to share with the Commission this work that has already been accomplished.

5. Our Desire to Interact with the Commission as It Moves Forward To Update Blood, Cell and Tissue Regulations

   A European Union-wide update and harmonization of human blood, cell and tissue regulation is greatly needed. It is an opportunity for the E.U. to lead the way for the entire world. CBA has the expertise, resources and enthusiasm to assist the Commission in our unique area of medicine and public health.

   Our aspiration is banking and manufacturing systems and regulations that provide optimal products and services for the benefit and safety of donors and patients, that are in harmony among national-jurisdiction laws and regulations, that encourage medical science innovation, and that enhance patient access to needed care.

Thank you for the opportunity to submit these comments. We hope that you find them useful. If you have questions, please do not hesitate to contact us.

We look forward to the possibility of working with you to enhance blood, cell and tissue banking and therapies.

Sincerely,

Joanne Kurtzberg, MD
President

Wolfgang Knirsch, PhD
Chair, European Section

Gesine Kögler, PhD
Secretary