

Legislative Hearing on 21<sup>st</sup> Century Cures on April 30, 2015  
Statement of Joanne Kurtzberg, M.D.  
President, Cord Blood Association

Subcommittee on Health  
Committee on Energy and Commerce  
U.S. House of Representatives

Chairman Pitts, Ranking Member Green and Members of the Subcommittee:

As President of the Cord Blood Association, I want to thank you for the opportunity to submit testimony for the record for your hearing entitled, “Legislative Hearing on 21<sup>st</sup> Century Cures.” I also am on the faculty of Duke University School of Medicine’s Department of Pediatrics and work as a Distinguished Professor of Pediatrics and Pathology. In addition, I am the Chief Scientific Officer of the Robertson Clinical and Translational Cell Therapy Program. I also serve as the Co-Director of the Stem Cell Laboratory and the Director of the Carolinas Cord Blood Bank. I have dedicated my professional career to cord blood research, banking and transplantation. Along with my fellow CBA members, I am seriously concerned that FDA’s licensure requirements for public cord blood banks are hampering innovation and restricting transplantation for treatment and cures, which is the subject of this written testimony.

### **Overview of the Cord Blood Association**

The Cord Blood Association (CBA) was created and incorporated over this past year. It is an international, non-profit organization that promotes public and private cord blood banking and the use of umbilical cord blood and related tissues for disease treatment and regenerative therapies. CBA’s members are public and private banks, as well as providers in the cord blood community and their patients. The CBA’s mission includes promoting the work of the cord blood community, saving human lives and changing medicine. Cord blood transplantation can treat and often cure some types of blood cancers and hereditary conditions, and recent studies have suggested that cord blood transplantation holds promise as a future treatment for other conditions, such as autism, traumatic brain injury, stroke and cerebral palsy.

### **Concerns Regarding FDA Regulation**

With regard to the 21<sup>st</sup> Century Cures draft that is being considered by the Energy and Commerce Committee, we want to raise an important issue that has caused difficulties for the cord blood banking industry. In 2014, the Food and Drug Administration (FDA) finalized licensure requirements for public cord blood banks to ensure the safety, purity and potency of cord blood units for transplantation. Although the CBA shares these critical goals, a number of these licensure requirements are more applicable for drugs than for cord blood units and impose significant cost and administrative burdens on cord blood banks. More importantly, these requirements threaten public health by stifling innovation and restricting growth of the national

cord blood inventory, which in turn limit access to life-saving transplantation for serious diseases and conditions.

The main issue with the regulation of cord blood banking relates to the application of current Good Manufacturing Processes (cGMPs), developed primarily for pharmaceutical manufacturers, to cord blood banking. The practical outcomes for such application include:

- Barriers to making improvements. The cGMPs delay the ability of banks to make timely adjustments to their processes, which may be necessary to enhance safety and effectiveness or to promote innovation.
- Duplicative validation. The FDA has required cord blood banks to validate a number of processes and products used in the process, although some are an unnecessary duplication of tests and validations.
- Burdensome environmental monitoring requirements. The preparation of cord blood is in no way comparable to the manufacture of pharmaceuticals in scale or scope; however, the FDA licensure requirements have not modified the environmental monitoring requirements to reflect this difference.
- Required creation of an expiration date. Prior to licensure, cord blood banks would test a unit to ensure high quality before use for transplantation. FDA now requires annual, regular testing of units, which leads to fewer units for actual patient use. At this time, there is no science indicating the shelf-life of stored cord blood. Therefore, it is difficult to understand why expiration dates are required in the labeling of these units. Units that are identified for therapeutic use should be tested before release but it makes little sense to annually test all units, especially when many cord banks have limited resources.
- Required stability testing. The cGMPs require cord blood banks to sacrifice units for stability testing, although such testing has not been shown to improve potency, integrity or sterility. Again, this testing leads to fewer units being available for patient use.

The sacrifice of units is problematic because, unlike a pharmaceutical, each unit of cord blood is biologically unique and cannot be replicated. A lost unit might have been the best biological match for a future patient.

## **Recommendations**

As described, the application of the cGMPs to cord blood units creates barriers to cord blood transplantation, and removal of such barriers is consistent with the intent of “The 21<sup>st</sup> Century Cures Act.” Thus, we request that you include language in the next iteration of this legislation to help resolve this issue.

Specifically, we recommend that Section 5021 in “Subtitle B—21<sup>st</sup> Century Manufacturing” be expanded to require the updated cGMPs specifically address good collection, storage, and maintenance practices for public and privately banked cord blood units, reflecting the cord blood banks’ real world experience with complying with current requirements. The cGMPs should be updated in collaboration with leading experts in the hematopoietic stem cell transplantation and cord blood banking communities in an open and transparent process. We would welcome the opportunity to work with you to develop specific legislative text to authorize this work.

## **Conclusion**

The CBA commends the Committee on its work on the 21<sup>st</sup> Century Cures legislation and it is our hope that as this legislation moves forward in the Energy and Commerce Committee, our concerns will be seriously considered in the final legislation. Mr. Chairman, thank you for the opportunity to submit written testimony on this important issue and we look forward to working with you, Members of the Committee and the FDA to ensure that these important issues are addressed in an appropriate manner.