NATIONAL MARROW DONOR PROGRAM®

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A Payor's Guide to:
Understanding FDA Cord Blood Regulation

To help you determine appropriate coverage for umbilical cord blood transplants, this document provides an overview of new FDA cord blood regulations. These regulations apply to cord blood donated to a public cord blood bank for use by patients who need a transplant from a source outside of their family (unrelated transplant).

Cord blood regulated as a biologic drug

Worldwide, more than 22,000 cord blood units (CBUs) have been shipped for use in hematopoietic cell transplantation through 2009.[1] The United States Food and Drug Administration (FDA) regulates this cell source as a biologic drug and began enforcement of licensure requirements in October 2011.

All CBUs are now categorized as:

- **licensed**—collected by a bank with an FDA-approved biologics drug license application (BLA) and meet licensure requirements
- **unlicensed**—collected before regulations were in place, collected by a bank without a BLA, or do not meet licensure requirements

Currently the worldwide CBU inventory for use in unrelated transplant is almost exclusively comprised of unlicensed CBUs; totaling more than 600,000 CBUs, most collected before regulations were in place. It is anticipated that international CBUs, as well as a large portion of U.S. historical cord blood inventory will always be unlicensed.

Unlicensed CBUs may be best treatment option

Prior to the licensure regulations, cord blood banks followed industry-accepted quality standards to collect and store CBUs for use in transplantation. It is important to understand that although CBUs may be categorized as "unlicensed", they still meet stringent quality standards and have been used successfully in transplant for nearly 20 years. More importantly, these units may be the best and/or only available match for a patient.

FDA enables access to unlicensed units through IND

The FDA recognizes the importance of unlicensed CBUs, and therefore, is allowing access to unlicensed CBUs for use in transplant through Investigational New Drug Application (IND) clinical research protocols.[2]

Since the vast majority of CBUs are unlicensed at this time, most patients that require a cord blood transplant will need to be enrolled in an IND protocol. CBUs distributed under an IND must meet strict FDA quality requirements. Those that do not meet FDA IND qualifications will not be distributed by the National Marrow Donor Program (NMDP).

NMDP holds IND for access to unlicensed CBUs

In an effort to help transplant centers access unlicensed CBUs for their patients, the NMDP is the sponsor of an IND protocol, 10-CBA.[3] The majority of transplant centers in the U.S. that perform cord blood transplants are participating in this protocol.

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If you have specific questions or need additional resources to support cord blood transplantation medical coverage, please contact the NMDP Payor Policy team at **NMDPPayorPolicy@nmdp.org**.

The NMDP was selected by the U.S. government to operate the nation's Cord Blood Coordinating Center as mandated by the Stem Cell Therapeutic and Research Act of 2005 and amended by Stem Cell Therapeutic Reauthorization Act of 2010. In this role, we educate patients, the public and medical professionals about cord blood transplantation.

References:

1. L. Foeken, World Marrow Donor Association, personal communication, January 2011
2. U.S. Department of Health and Human Services Food and Drug Administration Center for Biologics Evaluation and Research. Guidance for Industry and FDA Staff. Investigational New Drug Applications (INDs) for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications. June 2011.
3. National Marrow Donor Program, 10-CBA_Protocol: A multicenter access and distribution protocol for unlicensed cryopreserved cord blood units (CBUs) for transplantation in pediatric and adult patients with hematologic malignancies and other indications.