Biologics Compliance

FDLI 6th Annual Enforcement and Litigation Conference

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FDA

U.S. Department of Health and Human Services

Food and Drug Administration

Today's Focus: Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps)

Hematopoietic stem/progenitor cells

*DeMarco**

- In September 2006, Charlene DeMarco, a former doctor of osteopathy and her co-conspirator Elizabeth Lerner, a.k.a. “Elizabeth Cooperman,” were convicted of all charges contained in an 11-count federal indictment: one count of conspiracy to commit mail and wire fraud, three counts of mail fraud, six counts of wire fraud and one count of money laundering.

*From FDA News Release , December 19, 2007

DeMarco (cont…)

- “Evidence showed that from October 2002 until November 2004, DeMarco and Lerner agreed to defraud amyotrophic lateral sclerosis (ALS)* patients and their families by claiming they could treat ALS patients with stem cell therapy, when they knew they could not. The defendants falsely told the patients and their families that DeMarco had previously received FDA approval to treat ALS.”

*commonly called Lou Gehrig’s disease

DeMarco (cont…)

- In September 2007, Charlene DeMarco, was sentenced to 57 months in prison, ordered to pay $32,190 in restitution to victims and a criminal fine of $7500.
- In December 2007, Elizabeth Lerner was sentenced to 33 months in prison, ordered to pay $35,390 in restitution to victims and a criminal fine of $7500.

The Other Side of the Coin

- The National Marrow Donor Program
  - Public program, relies on unrelated allogeneic donors
  - Hematopoietic stem cells obtained from peripheral blood or cord blood are available to any patient
  - Registry of potential peripheral blood stem cell donors
  - Registry of cord blood units
  - Searchable to match donor or unit to recipient for hematopoietic reconstitution in patients with hematological malignancies
  - “Draft Guidance for Industry: Minimally Manipulated, Unrelated, Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution in Patients with Hematological Malignancies,” issued 1/16/07

Private Banking: Wave of Future?

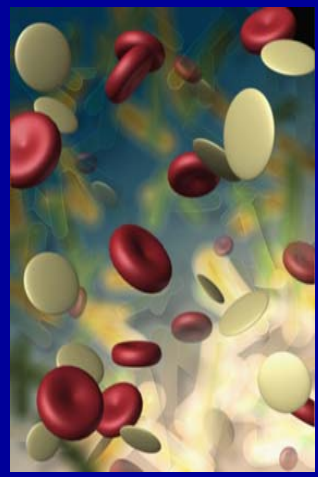
- Autologous – you pay to bank for future, *possible* need
  - Cord blood – potential use for siblings too; also baby teeth
  - Adult stem cells – different sources advertised

The New York Times

Questioning the Allure of Putting Cells in the Bank

By ANDREW POLLACK

Published: January 29, 2008



FDA Regulations

- Establishments that collect, process and store (the bank) and potentially distribute, are manufacturing HCT/Ps and, if the use is autologous and/or 1st and 2nd degree family related allogeneic, are regulated under section 361 of the Public Health Service Act and 21 CFR part 1271.

Concern

- Maintenance of proper conditions during long-term storage
- Banks promote that these HCT/Ps will cure or mitigate diseases (e.g. Parkinson's Disease), and these claims are unproven
- The manufacturer's objective intent is one of the factors taken into account in determining the level of regulation of HCT/Ps
- Such promotion might result in a determination that the HCT/P be regulated as a biological product under section 351 of the PHSA
- A biological product must be distributed under an FDA-monitored IND, or the manufacturer must prove to FDA that the product is safe and effective, and be licensed

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