Unapproved Regenerative Medicine Therapies: FDA Regulation and Concerns

Rachael F. Anatol, Ph.D., Deputy Director, Office of Tissues and Advanced Therapies, CBER, FDA and Melissa J. Mendoza, J.D., Deputy Director, Office of Compliance and Biologics Quality, CBER, FDA

FDA Regulation

The US Food and Drug Administration (FDA) regulates regenerative medicine therapies intended to treat or cure diseases or medical conditions. These products generally require FDA licensure/approval to be distributed or marketed to consumers. Before licensure, clinical trials of these products require FDA oversight.

The Problem

Manufacturers, suppliers, distributors, and healthcare providers continue to broadly market unapproved regenerative medicine therapies to patients. These products have risks but are often illegally marketed as being safe and effective for the treatment of a wide range of diseases or conditions, even though they haven't been adequately studied to demonstrate the claims of safety and effectiveness.

These products are not without risk. Safety concerns with any illegally marketed regenerative medicine therapy include:

- Blindness;
- Tumor formation;
- Neurological events;
- Bacterial infections including life-threatening blood infections;
- Reactions at the site of collection and administration;
- Unwanted inflammatory or immune response to the cell or therapy;
- Cells moving to another part of the body and turning into an unintended type of tissue or excessively growing in the body (i.e., forming a tumor);
- Failure of the therapy to work as anticipated when approved treatments are available;
- Cross-contamination with bacteria, viruses or mold related to processing (preparation of the product) or the therapy not being tested for infectious diseases such as hepatitis and HIV.

What Unapproved Therapies Are We Talking About?

- Stem cells
- Stromal vascular fraction (fat-derived cells)
- Umbilical cord blood and/or cord blood stem cells
- Certain placenta/chorion/amniotic membranes
- Umbilical cord products
- Amniotic fluid
- Wharton's jelly
- Exosomes
- Birth tissue/birth waste

What Diseases or Conditions?

None of these products have been approved for the treatment or prevention of:

- COVID-19, acute respiratory distress syndrome (ARDS), or any other complication related to COVID-19
- Orthopedic conditions, such as osteoarthritis, tendonitis, disc disease, tennis elbow, back pain, hip pain, knee pain, neck pain, or shoulder pain.
- Neurological disorders, such as multiple sclerosis, amyotrophic lateral sclerosis (ALS; Lou Gehrig's disease), Alzheimer's disease, Parkinson's disease, epilepsy, or stroke.
- Cardiovascular or pulmonary (lung) diseases, such as heart disease, emphysema, or chronic obstructive pulmonary disease (COPD)
- Autism
- Macular degeneration
- Blindness
- Chronic pain
- Fatigue
- And More!

Red Flags

- If any of these products are offered for sale
- Marketing statements that a product or establishment is registered and/or listed under Part 1271
- Marketing statements that a product is "minimally manipulated" or "intended for homologous use" such that it is compliant with FDA's regulations
- Any information to suggest that the products are being made available to patients outside of a clinical trial
- If you are referred to clinicaltrials.gov, please note that inclusion of a product in the clinicaltrials.gov database does *not* mean that it complies with the law

Contact FDA

We want to hear from you when a CBER regulated product is being used or marketed inappropriately, or if you have questions.

- **Consumers or Healthcare providers:** Contact OCOD@fda.hhs.gov,
- 800-835-4709 or 240-420-8010
- **Industry representatives or staff:** Contact Industry.Biologics@fda.hhs.gov, 800-835-4709 or 240-402-8020

What to Do?

- **Patients:** Ask if the FDA has reviewed the treatment, ask for the FDA-issued IND number, and ask to review the FDA communication stating the IND can proceed.
- **Healthcare Providers:** Manufacturers may file requests with FDA to determine how their products are appropriately regulated and FDA provides written feedback of its recommendation/determination. HCPs should ask the manufacturer of regenerative medicine therapies for a copy of this written feedback from the FDA regarding how the specific product is appropriately regulated.

Additional Information

Important Patient and Consumer Information About Regenerative Medicine Therapies | FDA
Questions and Answers Regarding the End of the Compliance and Enforcement Policy for Certain Human Cells, Tissues, or Cellular or Tissue-based Products (HCT/Ps) | FDA
Consumer Alert on Regenerative Medicine Products Including Stem Cells and Exosomes | FDA
Reporting Adverse Events Related to Stem Cells, Exosomes, or Other Products Marketed as Regenerative Medicine Products | FDA
FDA Warns About Stem Cell Therapies | FDA
Public Safety Notification on Exosome Products | FDA
FDA sends warning to companies for offering unapproved umbilical cord blood products that may put patients at risk | FDA
Advancing the Development of Safe and Effective Regenerative Medicine Products | FDA