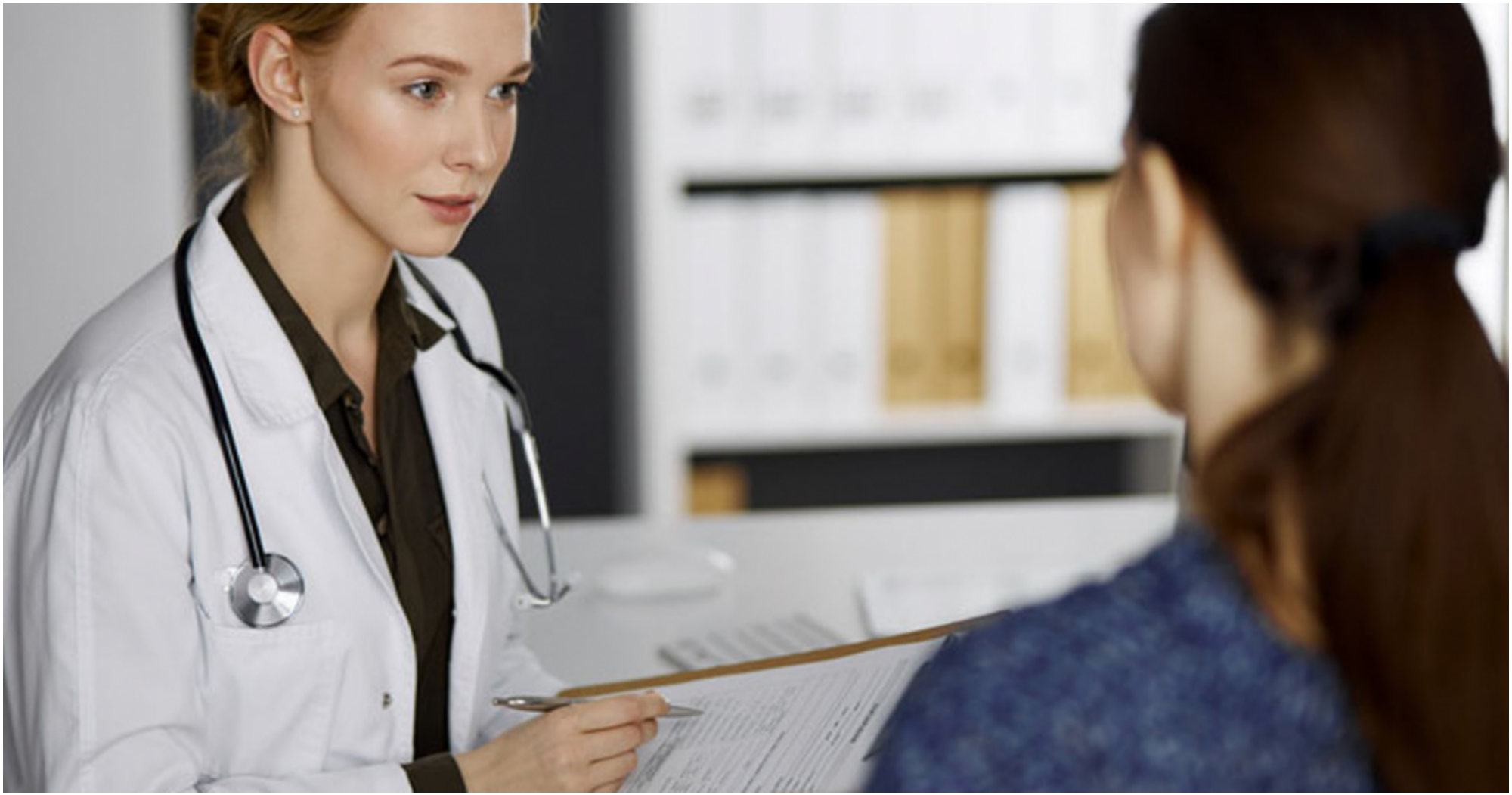
Potential Risks of Treatment with Unapproved Regenerative Medicine Products



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Protecting patients is at the core of what we do at the U.S. Food and Drug Administration. Now the FDA is providing additional resources to help consumers understand the risks associated with unapproved stem cell, exosome, and other products marketed as regenerative medicine products. The agency is also facilitating the reporting of side effects that may occur after their use. The FDA's receipt of this information is

important to developing a better understanding of the risks associated with these products and to helping the agency identify those clinics and manufacturers putting patients at risk.

Several times in recent years, the FDA warned consumers who have been treated with, or who are considering use of, unapproved stem cell, exosome, or other products marketed as regenerative medicine products, about their potential risk. These illegal products are often marketed by clinics under the umbrella term of regenerative medicine as being safe and effective for treatment of a wide range of diseases or conditions (e.g., Alzheimer's disease and other neurologic disorders, orthopedic conditions), even though they have not been adequately studied in clinical trials. And more recently, some of these clinics have been marketing or distributing their unproven products to treat complications related to COVID-19 – claims that are not based on adequate clinical data.

Consumers may also have been told by clinics and health care providers that because some of these products are made from their own cells, the FDA does not need to review or approve the treatment. That is simply not true. Further, claims that a clinic's FDA registration or inspection equates to FDA approval or a form of FDA endorsement, or that listing a "clinical study" on clinicaltrials.gov means that it has been reviewed and allowed to proceed by the FDA, are also false.

It is of utmost concern that unapproved regenerative medicine products that have no proven clinical benefit and that may cause serious harm are marketed to patients. Using these products may lead to delays in getting a proper diagnosis and could also discourage patients suffering from serious illnesses from receiving safe and effective treatments that may be available. What's also concerning is that we know that because these products are not FDA-approved, it's very likely that adverse events are underreported by health care providers who treat patients with these products, and by patients who may have been harmed. We therefore strongly encourage patients and their family members to ask questions before receiving these types of products – has the product been approved by the FDA? Has the product been approved for this use? Is the product being studied in a clinical trial that has been reviewed and allowed to proceed by the FDA? Am I being charged for an unapproved product?

We encourage patients and their health care providers to report to the FDA any potential adverse events and complaints related to the use of these products using the FDA's MedWatch Adverse Event Reporting program (/safety/medwatch-fda-safety-information-and-adverse-event-reporting-program). Stem cell, exosome, or other products marketed as regenerative medicine products may have the potential to treat many medical conditions and diseases. But for almost all these products, it is not yet known whether each product has any benefit — or if the product is safe to use. We want to make sure patients understand these potential risks in order to protect themselves from being treated with and paying for an unproven and illegally marketed products. The FDA has posted a webpage (/vaccines-blood-biologics/consumers-biologics/consumer-alert-regenerative-medicine-products-including-stem-cells-and-exosomes) for consumers that provides information about products marketed as stem cells, exosomes or other regenerative medicine products, including the conditions for which they are approved, and which products are not approved at all. The webpage also provides information on how to submit information about adverse events to the FDA's MedWatch Adverse Event Reporting Program.

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Unfortunately, we've found numerous instances of products being marketed to patients without FDA approval, as well as products being manufactured with significant deviations from current good tissue practice (CGTP) and/or current good manufacturing practice (CGMP) requirements. These violations have included deficient donor eligibility practices, and inadequate aseptic practices to prevent product contamination – violations that have the potential to place patients at risk. Patients have been harmed after being administered some of these violative products.

We encourage consumers who are considering treatment with regenerative medicine products to work with their health care providers to learn about the treatment being offered, and to visit our webpage (/vaccines-blood-biologics/consumers-biologics/consumer-alert-regenerative-medicine-products-including-stem-cells-and-exosomes). It is important to ask questions and understand the potential risks of treatment with unapproved products. The FDA will continue to work with the manufacturers of these products to support their development. But we will also continue to take appropriate action against those who put patients in harm's way.

Additional Resources:

- Consumer Alert on Regenerative Medicine Products Including Stem Cells and Exosomes (/vaccines-blood-biologics/consumers-biologics/consumer-alert-regenerative-medicine-products-including-stem-cells-and-exosomes)
- MedWatch Consumer Voluntary Reporting (Form FDA 3500B) (/media/85598/download)
- FDA Extends Enforcement Discretion Policy for Certain Regenerative Medicine Products (/news-events/press-announcements/fda-extends-enforcement-discretion-policy-certain-regenerative-medicine-products)
- FDA Consumer Update – FDA Warns About Stem Cell Therapies (/consumers/consumer-updates/fda-warns-about-stem-cell-therapies)
- JAMA Viewpoint – Identifying the Risks of Unproven Regenerative Medicine Therapies (https://jamanetwork.com/journals/jama/fullarticle/2767586) (http://www.fda.gov/about-fda/website-policies/website-disclaimer)