

Stem Cells Q&A with Daniel J. Weiss, MD, PhD

In just a few short years, stem cell clinics have opened and rapidly spread across the United States and around the world. There were just two stem cell clinics nationwide in 2009; today there are more than 700. As this new field of available medical therapies expands, it is more important than ever to be an informed consumer. To better understand stem cell treatments and to ensure we are asking our medical providers the best questions as they relate to stem cell therapy, we spoke with Dr. Dan Weiss from the Vermont Lung Center at the University of Vermont.

THANKS FOR JOINING US, DR. WEISS. CAN YOU PLEASE BEGIN BY DESCRIBING YOUR RESEARCH AND THE WORK OF YOUR CAREER THUS FAR IN THIS FIELD?

Dr. Weiss: I have had a longstanding interest in lung repair and regeneration after injury, notably gene and cell therapy approaches for lung diseases. Recent published work has included several benchmark publications that have included the first ever trial of cell therapy for emphysema and that have helped define a platform for studying stem cell-based therapies for lung diseases. I have also instituted a biennial meeting held at the University of Vermont, Stem Cells and Cell Therapies in Lung Biology and Diseases, that is widely viewed by the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), and non-profit respiratory disease foundations as the major meeting in the field. My overall goal is to provide a firm scientific basis for clinical application of cell therapies in lung diseases.

HOW ARE STEM CELLS USED IN MEDICINE?

Dr. Weiss: It's important to understand that there are different types of stem cells, and they can do different things that might be beneficial to patients. One particularly exciting action is in stimulating the body's own repair mechanisms to restore body tissues impacted by illness or injury. There is a wealth of research currently underway to study these and other possible uses of stem cells. However, we are still many years away from proven, FDA-approved clinical use.

Unless we are talking about specific stem cell therapies for certain cancers, such as leukemias and lymphomas, current stem cell clinics in Vermont are not providing any FDA-approved research or treatments. In an effort to recruit customers at their information sessions, staff from stem cell clinics assert that FDA certification has not been granted for their therapies due to the heavy lobbying of pharmaceutical companies. In actuality, FDA approval has not been granted due to the lack of clinical trials with stem cell therapies; here at the two stem cell clinics that have opened in Vermont, patients are not receiving any stem cell treatments that have been FDA-approved to be safe and efficacious.

A statement from the FDA in April 2019 notes: "For several decades now, stem cells obtained from the bone marrow or peripheral blood have had an established role in providing valuable treatment for patients with certain cancers, like leukemia, and certain other serious diseases and conditions, such as aplastic anemia. But most forms of regenerative medicine are still in early stages of development, and adult stem cells and stem cells from birthing tissues have not yet been shown to be safe and effective for use in the treatment of any other diseases or conditions."



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IF THESE TREATMENTS ARE NOT YET READY FOR CLINICAL USE, HOW ARE THESE STEM CELL CLINICS OPERATING?

Dr. Weiss: Over the past few years, a growing number of stem cell clinics have opened, which offer treatments that are not yet scientifically proven or reliable and that have not been rigorously studied in clinical trials. The clinics do this by utilizing loopholes in current FDA regulations, something the FDA is actively working to prohibit. In order to operate within these loopholes, it is unlikely that enough stem cells to offer a meaningful treatment are present. The FDA regulates cell and tissue products, but stem cell treatments that only use a patient's own minimally manipulated cells (which have not been manipulated, cultured, or had any other substances added in a laboratory) do not require FDA approval. The only FDA-approved stem cell therapies are those involving treatments for certain cancers and aplastic anemia, as previously mentioned. Some stem cell treatments may be dangerous; stem cell treatments offered at these clinics are often very different from those being studied through legitimate research taking place at universities and academic medical centers throughout the country.

WHY WOULD PEOPLE TURN TO THESE CLINICS FOR TREATMENT?

Dr. Weiss: Some patients with chronic or end-stage diseases turn to stem cell therapy, even if these treatments are still scientifically unproven, because they are motivated by the hope of a possible cure. These clinics unfortunately take advantage of these situations and charge very high prices; stem cell treatments can cost upwards of thousands of dollars out of pocket. Stem cell treatments are not covered by private insurance, Medicare, or Medicaid. Insurance companies currently view stem cell procedures as experimental. Further, these clinics offer misleading information about potential efficacy that is confusing to patients and to caregivers.

WHAT INFORMATION MAY STEM CELL CLINICS PROVIDE PATIENTS WITH? CAN THIS INFORMATION BE MISLEADING?

Dr. Weiss: The FDA does not approve, or decline to approve, stem cell clinics, but the FDA does have regulatory authority over the medical devices used by these businesses, as well as the cell-based products they sell. It is important to remember that cell-based products and treatments these stem cell clinics promote are not FDA-approved. Stem cell clinics are for-profit entities. The information they distribute to potential patients about possible benefits and treatments may sound very convincing. Discuss this information with a specialist in the specific field of medicine you are considering.

WHAT INFORMATION SHOULD THE STEM CELL CLINIC BE ABLE TO PROVIDE?

Dr. Weiss: Patients interested in possible stem cell treatment should receive: a detailed description of the treatment, the science that supports it, the expected outcome of the therapy, and any risks associated with the therapy. In many cases, particularly in a clinical trial, interested patients should be provided with a patient information sheet and informed consent documents that answer many of these anticipated questions. Patients should never hesitate to ask for more information or further explanation. The medical team involved should be knowledgeable of the medical condition, other treatment options, and the evidence that the treatment they are offering will be safe and that it will work.



WHAT ADVICE WOULD YOU OFFER SOMEONE CONSIDERING STEM CELL TREATMENT?

Dr. Weiss: I would encourage patients and family members to work closely with their health care providers to find out as much information as possible. There are several excellent web-based resources, notably those run by the two leading stem cell organizations, the International Society for Cell and Gene Therapy (ISCT, www.celltherapysociety.org) and the International Society for Stem Cell Research (ISSCR, www.isscr.org) which provide very useful information and, most importantly, a series of questions to ask if someone is considering stem cell therapy. These include, but are not limited to:

- Where do the cells come from?
- Are they actually stem cells?
- What kind of patients have been treated?
- What kind of outcomes have occurred?
- Have there been any safety issues?
- What are the risks?

Many stem cell clinics may not give straightforward answers to these questions, may magnify purported benefits, and may minimize potential risks.

WHOM ELSE SHOULD THE PATIENT SPEAK WITH?

Dr. Weiss: It is always best to get multiple opinions. The questions you asked and answers you received from the stem cell provider are best discussed with a trusted physician familiar with your condition, who can help you understand the treatment and your many options. It is a good idea to seek medical advice independent of the stem cell treatment provider to help assess whether the treatment and outcome claims offered are reasonable.

WHAT DOES A STEM CELL TREATMENT COST?

Dr. Weiss: Stem cell treatments can cost upwards of thousands of dollars out of pocket. Stem cell treatments are not covered by private insurance, Medicare, or Medicaid. Insurance companies currently view stem cell procedures as experimental.

WHAT SHOULD PATIENTS CONSIDERING STEM CELL TREATMENTS BE CAUTIOUS ABOUT?

Dr. Weiss: Often, stem cell clinics include patient testimonials in their advertising materials; patients who have been treated with expensive stem cell therapy want to believe that the intervention will have a positive effect, and therefore speak positively no matter the outcome. It is important to be aware when viewing this kind of advertising.

Ideally, every patient should be treated by a physician who is a specialist for that particular disease or condition. Risks should be explained clearly, and the process should be easily documented. Take note if the practitioner says there are no risks associated with the procedure. All medical treatments involve risk, and information should be readily available about the stem cell therapy you are considering. Just like any other medical decision you would make, take the time to consider all of your available options.

