

Patients Beware: Commercialized Stem Cell Treatments on the Web
A Report by the ISSCR Task Force on Unproven Stem Cell Treatments

Executive Summary

In this report, the International Society for Stem Cell Research (ISSCR) Task Force on Unproven Stem Cell Treatments summarizes their discussion and recommendations. Specifically, we recommend the development of web-based resources that: 1) include listings of individuals, clinics or other entities offering asserted stem cell therapies and whether they do or do not provide information showing that appropriate safeguards are in place; 2) resources that explain fundamental scientific principles of stem cell biology and the implications for stem cell treatments, and outline the widely accepted process of clinical translation; and 3) provides questions that a patient and/or caregiver should ask purveyors of asserted stem cell treatments to aid them in making treatment decisions.

The rapid advances in stem cell biology research and the application of this knowledge to experimental regenerative medicine have provided much hope that stem cells will offer new therapies for many serious diseases. There have been great advances in treating diseases and conditions of the blood system using blood-forming stem cells, and these show us just how powerful stem cell therapies can be. The transplantation of blood forming stem cells derived from bone marrow, mobilized peripheral blood, and umbilical cord blood have allowed many patients to regenerate blood formation when their own blood forming systems are damaged as a secondary effect of curative intent cancer therapies. These blood stem cell-containing populations have also saved the lives of patients with genetic or acquired blood disorders. In addition, some bone, skin and corneal diseases or injuries can be treated with grafting of tissue that depends upon stem cells from these organs. Some of these therapies have also been shown to be safe and effective. Currently the discovery of new stem cell treatments are moving toward and through clinical trials, although few are yet at the stage of approved clinical or commercial therapies. Advances in stem cell research continue to expand the scope of potential applications, however, many challenges lie ahead in taking this research and turning it into safe and effective treatments.

Before being marketed or adopted as standard of care, the ISSCR and other responsible scientific and medical organizations have consistently recommended that all new treatments should be tested in a careful and well-prescribed way that has been developed—and in many countries adopted into law—to protect patients' rights and safety. In most situations, this means advancing through phased clinical trials; starting with a very small number of people to check safety, and as the safety and side-effects are better understood and delivery method and dose are improved, gradually increasing the numbers of people to test the new treatment against existing approaches. During this process each step should be subjected to review by people otherwise unconnected with the trial. In a responsible center this means a careful review of the justification to use the approach to treat a specific disease or condition, methods

and provision of clinical follow-up; this review should be conducted by a group of people, who together have broad expertise and experience in research, medicine and ethics (often called an Ethics Review Committee or Institutional Review Board). Official regulatory agencies such as the European Medicines Agency (EMA) or the U.S. Food and Drug Administration (FDA) have the mandate to protect patients' rights at several stages in the progression of the trials to commercially available medicine and to ensure that any claims that a treatment will work are factual.

The excitement surrounding the capacity of stem cells to regenerate tissues or organs in experimental systems has led to a blossoming of clinics and practitioners claiming to deliver stem cells to treat conditions that are often untreatable by other means. The ISSCR is concerned that stem cell therapies are being offered to patients and their families around the world without credible scientific rationale or safeguards in place to ensure safety or expected efficacy. Without these safeguards, patients may be put at unnecessary risk, and legitimate progress towards new applications in clinical medicine may be jeopardized. Furthermore, in some situations, large amounts of money are being charged for apparently unsubstantiated therapies, a further departure from widely accepted norms. In a formal clinical trial setting it is not common practice for the provider to charge for the experimental treatment, rather costs of the experimental treatment and trial are often defrayed by the company developing the treatment or by local or national government funding.

To address these concerns, the ISSCR convened a Task Force on Unproven Stem Cell Therapies to formulate recommendations for the development of a web-based resource for patients, their advocates, clinicians and associations in evaluating claims of benefit from advertised stem cell therapies. In particular, the Task Force was asked to focus on a process for listing clinics or programs that did not meet minimum standards of assessing safety and efficacy before offering treatment, and to define criteria that might be used to systematically evaluate clinics or programs for inclusion on such a list.

The Task Force identified key elements that help patients and their caregivers determine if appropriate oversight and other patient protections are in place. These include: a) evidence that a medical ethics committee is involved to protect the rights and the safety of the patient; and b) evidence of supervision by an official regulatory authority such as the European Medicines Agency (EMA) or the U.S. Food and Drug Administration (FDA) for the trial of, or use of, a particular treatment for a particular disease.

The Task Force discussed the importance of researchers and clinicians reporting their findings and subjecting them to the scrutiny of independent experts in the field. They also reiterated previous reports from the ISSCR and other international bodies that trialing new medicines in patients is only justified when compelling scientific support is available, and that clinical testing should always be subject to rigorous and independent scientific and ethical oversight.

To provide a resource for people who are seeking help as they decide whether to put their time, money and safety into a stem cell therapy, the Task Force recommended the provision of the following tools that together, should allow people to judge whether the approach taken by a clinic of interest might lack the scientific rigor, transparency, and independent oversight

and regulation the ISSCR advocates. In providing these tools, the ISSCR does not ‘approve’ clinics, nor judge the degree of relative safety or effectiveness of treatments. Likewise, the ISSCR does not judge how well a patient’s rights will be protected, simply that there is evidence of oversight in place.

First, patients deserve to be alerted where claims of efficacy are unsubstantiated or where there are concerning departures from widely accepted practice. The Task Force recommends the inclusion of educational resources that explain fundamental scientific principles of stem cell biology and the implications for stem cell treatments and that outline the widely accepted process of clinical translation.

Second, the site should include further questions a patient (if possible working with their caregiver) should ask as they consider and evaluate a clinic and treatment. The list of questions will support a patient in assessing the forthrightness of the clinic alongside the clinical environment and long-term continuity of care.

Third, the ISSCR will develop listings of individuals, clinics or other entities offering asserted stem cell therapies and whether they do or do not provide the ISSCR on request with evidence that appropriate oversight and other patient protections are in place for the treatment(s) they offer. A series of simple questions will be initiated for clinics that come to our attention that claim to offer stem cell treatments for diseases or conditions outside of the blood system, skin stem cell treatments for burns, or corneal repair.

With these resources, the ISSCR seeks to arm patients, their families, physicians or other caregivers with information they need to make decisions about stem cell therapy. Take a closer look at stem cell treatments:

www.closerlookatstemcells.org

The International Society for Stem Cell Research is an independent, nonprofit organization established to promote and foster the exchange and dissemination of information and ideas relating to stem cells, to encourage the general field of research involving stem cells and to promote professional and public education in all areas of stem cell research and application. The ISSCR is committed to ensuring the promise of stem cell research is delivered to patients in a safe, effective and fair manner. In 2008, the ISSCR released “Guidelines for the Clinical Translation of Stem Cells” that call for rigorous standards in the development of stem cell therapies and outlining what needs to be accomplished to move stem cells from promising research to proven treatments. The ISSCR is not supported by government regulatory agencies. The members of the Task Force on Unproven Stem Cell Therapies received no fees or honoraria for their time spent to develop and publish this report.