



SBA STEM CELL POLICY

Interest in the potential use of stem cells to treat a variety of diseases and disorders, including Spina Bifida, are increasing. There are benefits and risks associated with the use of stem cell therapy (sometimes called regenerative medicine) in treating and conducting research in a variety of health conditions.

Many different types of stem cells exist. These include embryonic stem cells that exist only at the earliest stages of development; and various types of 'tissue-specific' stem cells (sometimes referred to as 'adult' or 'somatic' stem cells) that are found in various tissues in our bodies. Stem cells are defined by two properties:

- First, they can 'self-renew,' that is they can divide and give rise to more stem cells of the same kind.
- Second, they can mature or 'differentiate' into specialized cells that carry out a specific function, such as in the skin, muscle, or blood.

The range of diseases for which there are proven treatments based on stem cells is still extremely small. Disorders of the blood, and immune system and acquired loss of bone marrow function can, in some cases, be treated effectively with blood stem cell transplantation. However, the benefits and risks associated with stem cell use in Spina Bifida are not researched well and lack evidence to decide the benefits of such treatments and the risks associated with their use.

Ethical, Responsible, and Scientifically Worthy Research

It is the Spina Bifida Association (SBA) policy to support ethical, responsible, and scientifically worthy human stem cell research to the extent permitted by law. Scientifically, ethically, and responsibly appropriate ways to conduct research and means that ensure patient safety and measure outcomes objectively include, but are not limited to:

1. *Assessment and control of risks to study participants*

SBA will support research that adequately assesses and controls risks associated for study participants. Special care must be taken to control all known risks and prioritize the physical, mental, and emotional health of the study participants.

2. *Objectively scientific valid evaluation*

SBA supports objectively scientific valid evaluation that is characterized as using scientific claims, methods and results. The claims, methods, and results of science should not be influenced by particular perspectives, value commitments, community bias, or personal interests, to name a few relevant factors. Results of studies are evaluated using standard recognized methodology and should be able to be replicated and applied to similar groups of people.

3. *Tracking measurable outcomes*

SBA supports studies that adequately collect and utilized specific, measurable, and realistic outcomes. Outcome measure studies describe implications of the proposed outcomes for study design, and enumerate issues of bias that may arise in incorporating the ascertainment of outcomes into observational research, and means of evaluating, preventing and/or reducing these biases. Development of clear and objective outcome definitions that correspond to the nature of the hypothesized treatment effect and address the research questions of interest, along with validation of outcomes or use of standardized patient reported outcome (PRO) instruments validated for the population of interest, contribute to the internal validity of studies. Attention to collection of outcome data in an equivalent manner across treatment comparison groups is also required. Use of appropriate collection and analytic methods suitable to the outcome measure and sensitivity analysis to address varying definitions of at least the primary study outcomes are needed to draw robust and reliable inferences.

4. *Peer reviewed competing applications*

SBA will support peer reviewed and approved studies. Peer review is defined as the process of subjecting an author's scholarly work, research, or ideas to the scrutiny of others who are experts in the same field. It functions to encourage authors to meet the accepted high standards of their discipline and to control the dissemination of

research data to ensure that unwarranted claims, unacceptable interpretations or personal views are not published without prior expert review.

5. Institutional Review Board (IRB) approval

SBA will support IRB approved research. IRB is an appropriately constituted group that has been formally designated to review and monitor biomedical research involving human subjects. An IRB has the authority to approve, modify or disapprove research. This group review serves an important role in the protection of the rights and welfare of human research subjects.

The purpose of IRB review is to assure, both in advance and by periodic review, that appropriate steps are taken to protect the rights and welfare of humans participating as subjects in the research. To accomplish this purpose, IRBs use a group process to review research protocols and related materials (e.g., informed consent documents and investigator brochures) to ensure protection of the rights and welfare of human subjects of research.

Retail Therapy

It is understandable that some people with Spina Bifida or their families would be interested in stem cell therapy in the hope of a cure or improved health. There are hundreds of clinics in the United States and around the world that continue to provide costly stem cell treatments and often market them as cures for a variety of diseases and conditions without sound scientific evidence or regulatory approval. These “retail therapy” treatments have, in some cases, caused patients great harm physically, and at great expense financially, and they are not supported or condoned by SBA.

Additional Information

For individuals who wish to understand more about stem cell therapy, please see the following website resource: <https://www.closerlookatstemcells.org/patient-resources/>

This policy supersedes any earlier policies in regards to stem cells and will apply to all policies and procedures of SBA.

Adopted by the SBA Board of Directors on March 29, 2020.