UNPROVEN STEM CELL INTERVENTIONS AND ENCOURAGING COLLABORATION BETWEEN REGULATORS AND DESPERATE PATIENTS WITHIN THE CLINICAL TRIAL PROCESS

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Introduction
Stem cells have been touted by scientists as the new future for medicine, but with limited therapies currently available, clinics around the world are marketing unproven interventions utilizing stem cells to allegedly “cure” diseases ranging from Autism to Multiple Sclerosis. The continued marketing and use of experimental stem cell–based interventions is problematic and unsustainable. Central problems include the lack of patient protection, regulation of clinical sites and clinician licensing. These interventions lack evidence of safety and efficacy; patients may be wasting money and time and they may be foregoing other opportunities for interventions that have not been shown to be safe and effective. The landscape of stem cell tourism should prompt a re-evaluation of current policy approaches to studying stem cell–based interventions with respect to the design, initiation and conduct of clinical trials in the U.S. as well as abroad. To understand this issue and ways to promote change, we reviewed and analyzed several national and local policy initiatives that tried to provide expanded access to patients.

Stem Cell Tourism
Many patients have become frustrated with the perceived slow pace of progress in regenerative medicine and stem cell research. Scientists and policy scholars are concerned that unproven stem cell–based interventions marketed in under-regulated areas of the world to attract patients—also known as stem cell tourism—harm both the patient and the field (ISSCR 2008). Stem cell (SC) tourism poses numerous concerns for patients, scientists and society:

- Patients may have inadequate information about risks— which are significant and include death—and the low possibility of benefit. The lack of oversight in many countries gives little confidence that we have an adequate understanding of risks.
- Patients face significant out-of-pocket costs, including travel expenses, and coverage for injuries resulting from the intervention may not be available.
- The lack of transparency regarding what interventions are provided and at what doses could make it difficult to receive proper medical attention in the future.
- Patient safety and liability standards in countries hosting SC tourists may be lower than what patients expect in their home countries.
- Failure to adhere to a protocol and collect data systematically makes it impossible to learn about the safety and efficacy of the interventions, undermining the interests of future patients and society.
- SC tourists often do not have long-term follow-up care, making it impossible to identify long-term risks.
- SC tourists may be ineligible to participate in legitimate research or to receive other cell–based interventions in the future.

SC Tourism: The Problems
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Developing Stem Cell Tourism Policy
Problems associated with stem cell tourism can be addressed as a matter of public policy by engaging relevant stakeholders—patients, researchers and regulators. National clinical trial oversight agencies, such as the FDA in the United States, should assess its current policy and look for ways to respond to patient concerns while fostering responsible clinical research.

Stakeholders and Policy Goals for Combating Stem Cell Tourism

Patients
- Respect the interests of seriously ill patients and the authority of individuals to make decisions about what therapies are reasonable for them
- Protect health and safety of all individuals as well as the public, as well as maximize potential benefits and minimize risks to patients and society to protect and promote health and safety
- Promote ethical and rigorous scientific research
- Foster well-placed trust in physicians and health care institutions

Regulators
- Protect and promote health and safety
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Researchers
- Respect the interests of seriously ill patients and the authority of individuals to make decisions about what therapies are reasonable for them
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Policy Development Goals
Public policy should be developed to bring together the major stakeholders, patients, regulators and researchers, to address SC tourism.

Policy Alternatives
New policies have been developed, or are under review, to help bridge between regulators, physicians, scientists and patients. While these new policies address patients’ concerns about access, they also increase overall risks to patients.

A. Japan’s “Pharmaceutical, Medical Devices and Other Therapeutic Products Act”
- Creates a conditional and time–limited pathway for regenerative medical products and allows for a shorter review process period.
- Reasonable likelihood of clinical benefit needs to be demonstrated.
- Approval for intervention must be obtained within a seven–year period or will be removed from the market.

B. UK’s “Saatchi Bill”
- Named after Lord Saatchi, the Medicines Innovation Bill in the UK was first proposed in 2012.
- Allows doctors to depart from the existing range of medical treatments after seeking advice from another qualified physician.
- Must get consent and comply with professional requirements such as registration of the treatment.
- Never passed the parliament.

C. U.S. “Right to Try” Model Legislation from the Goldwater Institute
- Expands patients’ right to try experimental drugs, devices or products after they have completed a phase I trial.
- To be eligible, patients need to be in the advanced stages of a terminal disease with no cure and must consult with a physician about all other available treatments and get written consent.
- The manufacturer is not required to make it available and can charge a manufacturing cost or offer it for free.
- Insurance is not required to pay for the treatment but can include coverage for the treatments.
- No licensing board can take any actions against a medical professional who has recommended or treated with a investigational drug, product or device.
- As of May 2016, 23 states have passed “Right to Try” legislation.
- While similar bills have been proposed in the U.S. Congress, none have passed.

Conclusion
Public policy should be developed to address stem cell tourism. Stakeholders, including scientists, clinicians, regulators and patient advocates, need to work together to find a compromise to encourage patients to stay in their home country and within the clinical trial process instead of seeking unproven treatments abroad. Without stakeholders working together, we will continue to see new legislation being reviewed and passed which tries to increase patient access but often increases their risk, as well.

References

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Further Information
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