ABSTRACT W-1540

UNPROVEN STEM CELL TREATMENTS AND RE-EVALUATING THE ROLE OF THE US FDA: BRINGING THE LOST AND DESPERATE BACK HOME

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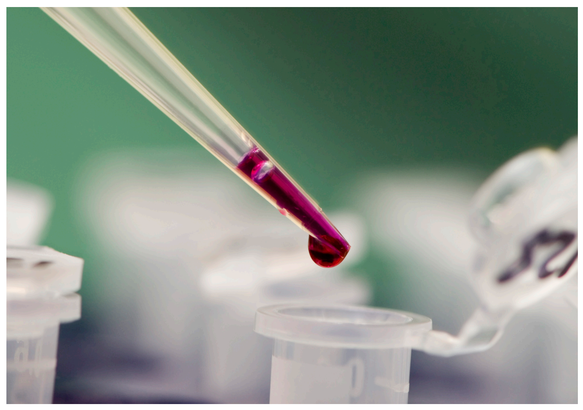
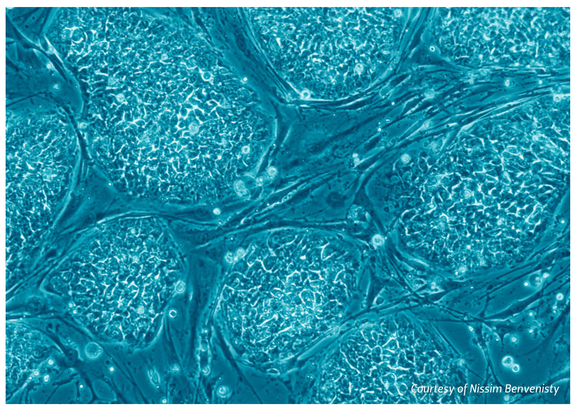
Introduction

In 2004, patient advocate groups were major players in promoting and helping to pass significant public policy and funding initiatives in stem cells and regenerative medicine. In the following years, advocates were also actively engaged in Washington DC, encouraging policymakers to broaden stem cell research funding, which was ultimately passed after President Barack Obama came into office. Advocates did this because they were told stem cell research would lead to cures. After waiting more than 10 years, many of these same patients are now approaching clinics around the world offering experimental stem cell therapies instead of waiting for scientists in the United States to go through clinical trials. How did the same groups who were once (and often still are) the strongest supporters of stem cell research now become the opponent? And how can scientists work to bring them back home? In this presentation, we argue that the marketing of experimental interventions is problematic. Public policy should be developed to correct this situation.





Courtesy of Nissim Benvenisty

Stem Cell Tourism

Many patients, who once were strong supporters of stem cell (SC) research, are frustrated by the perceived slow pace of progress. In a response to marketing efforts, they have become SC tourists. Scientists and policy scholars are concerned that unproven SC interventions may harm patients and the field and have warned of the need to pursue rigorous studies (ISSCR 2008). The US Food and Drug Administration (FDA) has asserted authority over interventions, even those involving a patient's own SC cells when those cells have been more than minimally manipulated. But this only led clinics to move overseas. SC clinics may be unable to obtain FDA permission to initiate trials because of limited data. All evidence suggests that SC tourism is alive and well.

Figure 1 — SC Tourism: The Problems

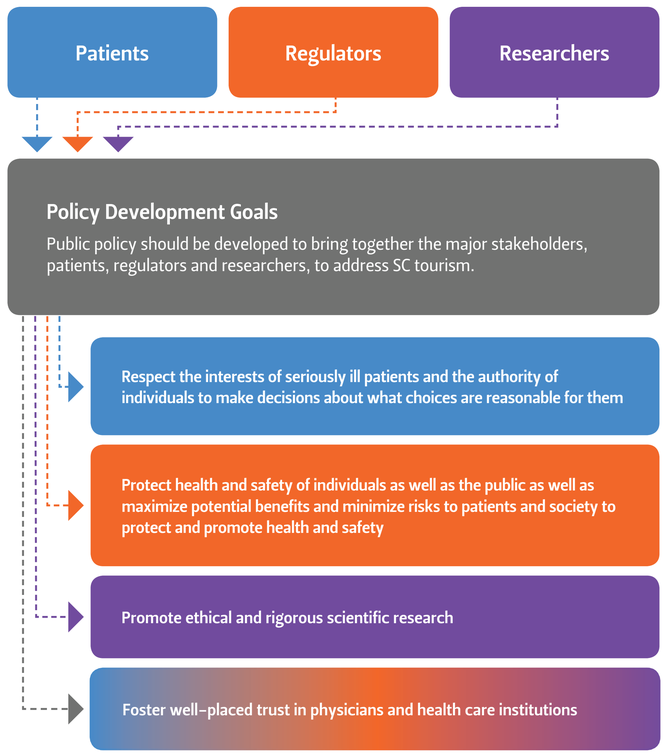
SC tourism poses numerous concerns for patients, scientists and society:

- Patients may have inadequate information about risks and the low possibility of benefit.
- Risks are significant. Deaths have been reported, and lack of oversight 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.
- 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 in the future or to receive other cell-based interventions in the future.

Developing Stem Cell Tourism Policy

The practice of more and more patients with debilitating illnesses going abroad to less developed medical infrastructures to seek SC treatment raises serious problems that should be addressed as a matter of public policy. To accomplish this, the US FDA should assess its current SC policy and look for ways it could make changes to bring patients home while fostering the responsible conduct of research.

Figure 4 — Stakeholders and Policy Goals for Combating Stem Cell Tourism



Conclusions

Public policy should be developed to address stem cell tourism. Central problems include patients lack protection of US liability standards, regulation of clinical sites, and clinician licensing. In addition there is inconsistent or non-existent follow up care and no assurance for patients that they are receiving the intervention promised or of what dosage they are receiving. Furthermore, these experimental interventions have insufficient evidence of safety and efficacy; patients may be wasting money and time and forgoing other opportunities by obtaining an intervention that has not been shown to be safe and effective. Finally, current practices do not contribute to scientific progress because the information from patients is not suitable for follow up research to measure outcomes. Using the HIV/AIDS and breast cancer advocate cases as examples, we identify key priorities and goals for this policy effort. The current landscape of cell-based interventions and stem cell tourism should prompt a similar re-evaluation of current approaches with respect to the design, initiation, and conduct of US clinical trials.

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Learning From the Past

SC interventions are not the first unproven interventions to which patients have sought access. Changes that emerged from HIV/AIDS advocacy work demonstrate the possibility of successfully using public policy to advance competing goals. In contrast, coverage of high-dose chemotherapy and autologous bone marrow transplantation (HDC/ABMT) for advanced breast cancer, demonstrates a public policy failure. Lessons from both cases can help shape the policy response to SC tourism.

Figure 2 — HIV/AIDS Activism in the 1980s: Accelerating Research and FDA Approval

AIDS activists in the 1980s collaborated with the FDA to develop more efficient and effective policies to accelerate access to new treatments. The FDA changed several policies in response, advancing the interests of patients, researchers, clinicians, and society.

- Investigational new drug program facilitated drugs access outside of trials (1987)
- Fast-Track program expedited approval of some new drugs (1988)
- Parallel-Track program allowed some patients ineligible for clinical trials access to experimental drugs (1990)
- Accepting surrogate endpoints accelerated drug approval (1992)

Figure 3 — HDC/ABMT Breast Cancer Advocacy Impaired Research and Harmed Patients

HDC/AMBT had been used successfully in some cancers and preliminary evidence suggested it might be effective in advanced breast cancer. After several lawsuits, many insurance companies started covering HDC/ABMT for women with breast cancer despite lack of safety and efficacy data.

- Women did not want to risk getting standard of care in randomized trials and refused to participate in research.
- Lack of participation in clinical trials delayed data collection
- Studies demonstrated that HDC/ABMT was ineffective in breast cancer and shortened life
- Patients received the ineffective and unsafe intervention and significant resources were spent
- Activism through the courts failed patients and society

Further Information

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Contact Information

The International Stem Cell Policy Program