

May 6th, 2017

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The Honorable John Zerwas
Texas House of Representatives
Room GW.17 – State Capitol
Austin, TX 78768

Regarding: House Bill 661, House Bill 810 and House Bill 3236

Dear Representative Zerwas:

The International Society for Stem Cell Research (ISSCR), the leading professional organization of stem cell science and regenerative medicine, opposes House Bill 661, House Bill 810 and House Bill 3236, which would allow investigational agents to be sold to patients without first providing rigorous evidence of safety and efficacy. This would put patients at serious risk of harm from unproven treatments.

Historical data show that most experimental therapies that initially look promising based on early clinical trials subsequently fail, proving to be either unsafe or ineffective in phase II and phase III clinical trials. This means that if the lower standards in these bills are enacted, many of the new treatments that would become available to patients would ultimately prove to be unsafe or ineffective. Patients have been [harmed](#) as a result of receiving unproven cell therapies from physicians in the United States, with serious outcomes including blindness and paralysis. These bills would cost more lives than they save and would be detrimental to the citizens of Texas.

The International Society for Stem Cell Research (ISSCR) represents more than 3,600 members in the United States and over 60 other countries. The ISSCR strongly opposes this legislation because it would lower standards for new regenerative medicine therapies.

Typically, investigational medical treatments are tested for toxicity in Phase I trials, preliminary evidence of therapeutic efficacy in Phase II, and for definitive evidence of safety and efficacy in carefully controlled Phase III clinical trials. This phased approach minimizes harm to patients during the testing process and ensures that physicians and patients have as much information as possible on the risks and benefits of these products. Patients deserve this full information before making what are often life or death decisions on which therapies to use. If Texas makes available products that have been tested only in Phase I clinical trials, these bills will expose patients to unsafe and ineffective therapies and will undermine confidence in Texas' medical system.

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In their current form, House Bill 661, House Bill 810 and House Bill 3236 will also allow companies to sell unsafe and ineffective therapies. It may sound like an appealing idea to allow seriously ill patients accelerated access to experimental therapies; however, in the absence of full clinical testing, these bills will allow snake oil salesmen to sell unproven and scientifically dubious therapies to desperate patients.

It is critical that any unproven stem cell-based intervention provided outside of a formal clinical trial has appropriate review and oversight. This review should include careful scrutiny of the potential risks and benefits of the intervention for the particular disease or injury, and of the informed consent process. Data should be collected on the safety and efficacy of the experimental therapeutics in all patients, and these data should be made broadly available. A medical standard of care cannot be developed without such information, leaving physicians unable to fulfill their duty to meet that standard. Neither physicians nor patients in Texas should be forced to make treatment decisions without information on the results that such treatments have achieved in other patients.

Finally, approval for marketing and reimbursement should remain conditional upon the completion of clinical investigations that demonstrate safety and efficacy, as judged by rigorous, independent, and expert regulatory review. Companies should not be able to make a profit by selling therapies that do not have FDA approval and whose safety and efficacy have not been established in phase III clinical trials. By allowing companies to market unproven therapies, the bill removes their incentive to invest in the research needed to validate safety and efficacy, and without this investment, the cost of treatment failure will be transferred to patients.

The ISSCR believes that, as currently drafted, House Bills 661, 810 and 3236 will put patients at risk. Ensuring the health and welfare of patients should be a top priority in the approval process, along with guarding against the sale of unproven therapies. The people of Texas deserve no less.

Sincerely,



Sally Temple, Ph.D.
President, ISSCR
Scientific Director, Principal Investigator and Co-Founder, Neural Stem Cell Institute, Rensselaer, NY 12144