5215 Old Orchard Rd, Suite 270
Skokie, IL 60077 USA
Tel: +1-224-592-5700
Fax: +1-224-365-0004
isscr@isscr.org
www.isscr.org

June 27, 2017

The Honorable Evandro Carvalho, Vice Chair
Joint Committee on Public Health
190th General Court of the Commonwealth of Massachusetts
24 Beacon Street, Room 136
Boston, MA, 02133

RE: House Bill No. 1129

Dear Representative Carvalho:

On behalf of the International Society for Stem Cell Research (ISSCR), the leading professional organization of stem cell science and regenerative medicine, I want to thank you for the opportunity to share our concerns about House Bill 1129, which would allow investigational agents to be provided to patients outside of the context of a clinical trial, without first establishing the safety and efficacy of the approach through rigorous clinical trials. The ISSCR, which represents more than 3,600 members in the United States and over 60 other countries, strongly opposes this legislation because it would lower standards for new regenerative medicine therapies.

Using unproven treatments on significant numbers of patients, particularly in a for-profit context, raises major ethical and integrity concerns and jeopardizes patient safety. The practices that would be permissible under House Bill 1129 are contrary to the guiding principles of the ISSCR 2016 Guidelines for Stem Cell Research and Clinical Translation. These guidelines help assure the integrity of stem cell science and its translation to medicine.

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.

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 Massachusetts makes available products that have been tested only in Phase I clinical trials, this bill could expose patients to potentially unsafe and ineffective therapies and will undermine confidence in the Massachusetts medical system.

House Bill 1129 will also allow companies to sell potentially 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, this bill 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 Massachusetts 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 House Bill 1129 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 Massachusetts deserve no less.

If the ISSCR can be of any assistance as you move forward on this critical issue, please contact me directly, or contact Kaye Meier on my staff (KMeier@ISSCR.org).

Sincerely,

Hans Clevers, MD, PhD
President, ISSCR
Professor of Molecular Genetics at Hubrecht Institute/University Medical Centre Utrecht