ISSCR INTERNATIONAL SOCIETY FOR STEM CELL RESEARCH

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March 7, 2019

The Honorable Senfronia Thompson
Chair, Public Health Committee
Texas House of Representatives
Room 3S.6
P.O. Box 2910
Austin, TX 78768

The Honorable John Wray
Vice Chair, Public Health Committee
Texas House of Representatives
Room E1.302
P.O. Box 2910
Austin, TX 78768

Dear Chair Thompson and Vice Chair Wray,

On behalf of the International Society for Stem Cell Research (ISSCR), I write to voice our opposition to HB 805, which would put patients at risk and undermine the effective U.S. FDA Expanded Access Program that already gives seriously ill patients access to experimental treatments. The ISSCR is the leading professional organization of stem cell scientists and represents more than 4,000 members in United States and around the world. Our members are scientists, clinicians, ethicists, and educators dedicated to the responsible advancement of stem cell research and its translation to the clinic. We believe HB 805 is unnecessary and interferes with an effective program already in place that ensures patient safety while providing accelerated access to experimental therapies.

Stem cell science and regenerative medicine hold enormous potential to yield new treatments for diseases that currently have no cure, including Parkinson's disease, multiple sclerosis, heart disease, and juvenile diabetes. This legislation will cause long-term harm to this nascent field and expose patients to unnecessary risk by facilitating the dissemination of therapies that have not been proven to be safe or effective.

Unfortunately, unscrupulous clinics in Texas are marketing unproven "stem cell therapies" directly to patients without demonstrating safety or effectiveness in clinical trials, in violation of FDA regulations. In many cases, we would not expect these fake stem cell therapies to benefit patients based on what we understand about the biology. Moreover, companies that are willing to ignore FDA regulations related to demonstrating safety and effectiveness often also ignore regulations promoting good manufacturing practices. Texans have been harmed by these products, for example as a result of pathogen contamination in unproven stem cell products. The Texas legislature should not encourage the sale of these fake therapies or encourage companies to evade FDA regulation.

Patients understandably seek experimental therapies when they have inadequate treatments for serious and incurable diseases. With the FDA's existing Expanded Access Program, also known as Compassionate Use, patients are able to access experimental treatments through a process that provides important checks and balances and ensures patient safety, facilitates drug development, and preserves the integrity of clinical trials. Importantly, this program is already available to patients with serious diseases that have "substantial impact on day-to-day functioning"[i]. Instead of bypassing the Expanded Access Program, Texas should consider increasing awareness and use of FDA's existing and effective program.

Over the last few years, the U.S. Congress, Texas, and other states have passed "Right to Try" bills that have sought to provide patients with alternative pathways for accessing experimental treatments. The ISSCR opposed these bills because we believed they undermined the FDA's ability to ensure the safety and effectiveness of the therapies that are sold to consumers. These regulations are necessary to protect the health and welfare of patients, to ensure the integrity of clinical trials, and to provide a clear and predictable path for new products to be developed and tested. Despite these new pathways, patients, physicians, and manufacturers have continued to choose to use the FDA's expanded access program because of its important checks and balances.

The proposed legislation (HB 805) would provide an additional unnecessary route for snake-oil salesmen to evade oversight from the Texas Medical Board and the FDA. If passed, this legislation would embolden unscrupulous actors and clinics selling unproven and scientifically dubious therapies to patients, a growing problem in the U.S. and Texas. According to a 2016 study,[ii] 71 of these direct-to-consumer clinics operated in Texas. As these clinics grow in Texas and beyond, they are exacting medical, emotional, and financial tolls on citizens. A number of Americans have already been blinded, paralyzed, and infected with dangerous pathogens from unproven stem cell treatments, some of which were marketed in a way that caused patients to believe they were participating in legitimate clinical trials or receiving scientifically validated treatments.

We urge you to reject HB 805 because it would undermine the existing and effective FDA program for providing access to experimental treatments and potentially create a loophole for unscrupulous clinics to market unproven products to patients. Thank you for considering our concerns regarding HB 805; if the ISSCR can clarify any of these views or be of assistance, please contact Eric Anthony, ISSCR's Director of Policy at eanthony@isscr.org.

Sincerely,

Doug Melton

Douglas A. Melton, PhD
President, ISSCR
Xander University Professor, Harvard University
Co-Director, Harvard Stem Cell Institute

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[i] Investigational New Drug Application, Title 21 CFR §312.300
[ii] Turner & Knoepfler, Cell Stem Cell, 2016 Aug 4;19(2):154-157