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Texas H.B. 810: Increased Access to Stem Cell Interventions or an Increase in Unproven Treatments?

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Born of the expectations and hype associated with regenerative medicine, there are now numerous clinics around the world selling stem cell-based interventions (SCBI) that have yet to be proven effective or safe, with little to no accounting of the outcomes being collected. In the United States, SCBI are overseen by the U.S. Food and Drug Administration (FDA), but several SCBI clinics have been pushing for policies to expand access and circumvent FDA oversight. Related to this effort, in 2017, Texas passed a bill, HB 810, which allows clinics to provide ''investigational stem cell treatments to patients with certain severe chronic diseases or terminal illnesses.'' In this article, we describe how the new law relates to another deregulation movement, state and federal, the Right to Try laws, the content of HB 810, and the state legislators' intent in passing HB 810.

Keywords: HB 810, Texas, unproven stem cell-based interventions, right to try, FDA

Stem Cell-Based Interventions and Emerging U.S. Clinics

Born of the expectations and hype associated with stem cells and regenerative medicine over the past two decades, there are now numerous clinics around the world selling stem cell-based interventions (SCBI) that have yet to be proven effective or safe, with little to no accounting of the outcomes being collected [1–3]. SCBI treatments might hold the key to help patients, but they also have serious risks of side effects, including graft-versus-host disease, unintended harm, and even cancer. Clinics are charging exorbitant prices, some for more than $20,000 per treatment [1,2].

In the United States, SCBI are overseen by the U.S. Food and Drug Administration (FDA) if the cells are more than ''minimally manipulated'' [4,5]. To fall outside of FDA regulation, manipulation must ''not alter the original relevant characteristics of the tissue relating to the tissue's utility for reconstruction, repair, or replacement,'' nor ''alter the relevant biological characteristics of cells or tissues'' [6]. But U.S. clinics have been pushing for policies to expand access and circumvent FDA oversight.

A part of the new effort to challenge the status quo is the Texas law, HB 810. This law, passed in 2017, allows clinics in Texas to provide ''investigational stem cell treatments to patients with certain severe chronic diseases or terminal illnesses'' [7]. In this article, we will describe how the new law relates to another deregulation movement—the Right to Try, the content of HB 810, and the legislators' intent.

"Right to Try" Laws

The expansion of unproven SCBI clinics in Texas is linked with a growing U.S. trend promoting medical freedom and choice, known as Right to Try. The initial impetus of the Right to Try movement was rooted in patients' dissatisfaction with the FDA's prolonged approval procedures and paperwork associated with their Expanded Access Program, as well as, arguably, an incomplete understanding of the clinical trial process [8]. The FDA's Expanded Access Program allows use of experimental drugs during phase 2 or phase 3 of clinical trials with approval from an ethical oversight board. Right to Try laws allow terminally ill patients to request access to experimental drugs as early as phase 1 and without FDA oversight or approval by an ethics oversight board [8].

Despite concerns raised by patient advocate groups and scientists, 40 states, including Texas, passed Right to Try laws (http://righttotry.org/in-your-state). In addition, on May 30, 2018, President Trump signed a federal version of Right to Try into law. Sen. Ron Johnson (R-WI) intended the law to ''diminish the FDA's power over people's lives, not increase it [9].''

Texas House Bill 810: Expanding Stem Cell Intervention Access

In 2017, state legislators brought forward an expansion of the state's Right to Try law—HB 810 or Charlie's Law—to allow access to ''investigational stem cell treatments to

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patients with certain severe chronic diseases or terminal illnesses''(named after late Texas Representative Charlie Howard who died of cancer in 2017) [7]. Two key authors of the bill were Republicans, Rep. Tan Parker and Sen. Paul Bettencourt. Sen. Bettencourt acknowledged that ''the 2015 law was the Right to Try law for those that were terminally ill. And the expansion that he [Rep. Parker] came up with was to move to the chronically ill,'' [10]. Sen Bettencourt said, ''I would rather have the state helping out with these therapies than waiting for the FDA to make up their mind in 20 years.''

Motivated by medical freedom and choice, Rep. Parker remarked, ''Why can't someone that is of age have the ability to sign off, so to speak, with regard to a proper medical release on the ability to do something that can make such a dramatic difference in their life, and their lifespan, and their quality of life?'' Furthermore, Rep. Parker believed the new law would help medical innovation. ''… with what we're doing with adult stem cell, we need to look for more innovation and allow the states to be the incubators, so to speak, of innovation'' [10].

As written, HB 810 requires that the SCBI ''[be] under investigation in a clinical trial and [be] administered to human participants in that trial; and has not yet been approved for general use by the United States Food and Drug Administration.'' Furthermore, similar to Right to Try bills, a physician must determine that there is no other FDA-approved alternative and that treatments are ''unlikely to alleviate the significant impairment or pain associated with the severe chronic disease or terminal illness.'' In addition, before treatment, a patient must sign a written informed consent [7].

A series of amendments to the bill added additional protections for patients. Sen. Charles Schwertner added an amendment that allows patients to sue physicians for unintended consequences of stem cell therapies. Stressing public safety, he said, ''I want to make sure that those individuals that go to see a physician, for instance … any sort of change, from regulatory standpoint, is to and in conjunction with that guiding light of protecting the public safety'' [10]. A second amendment by Sen. Van Taylor requires treatment be administered directly by a physician and ''overseen by an institutional review board [IRB]'' [7]. This Institutional Review Board (IRB) is required to be affiliated with an academic medical school or a hospital with a minimum of 150 beds, ~25% of total acute care hospitals in the state, limiting access to larger hospitals in Texas with more expertise (www.dshs.texas.gov/chs/hosp/hosp2.aspx). Rep. Garnet Coleman remarked that these amendments were best for state health. He wanted ''to make sure, as a state, that when an individual walks into either a research study or walks into a provider's office or any of those circumstances that there are a set of rules that everybody lives by that encourages the therapy, but at the same time makes sure that we're learning from the use of that therapy moving forward.''

In addition, Sen. Taylor's amendment required the IRBs to submit annual reports to the Texas Medical Board. IRBs are asked to keep records on all patients, the treatment given, and the effect of the treatment. This information will be the basis of the annual report that is required to be publically available.

Concerns

There are numerous concerns about the new legislation. HB 810 allows experimental SCBI only if the patient has a ''severe chronic'' or ''terminal'' illness, but unfortunately, there is no clear guide on what types of illnesses are included in the severe chronic category. This ambiguity was reflected by the law's description of severe chronic disease as ''a condition, injury, or illness that: (A) may be treated; (B) is never cured or eliminated; and (C) entails significant functional impairment or severe pain'' [7]. The Texas Department State Health Services (DSHS) must determine what is and is not included. DSHS held a stakeholder meeting in April 2018 to accept comments and suggestions, but is still in the process of developing rules to designate medical conditions as chronic or terminal.

In addition, the reporting requirements are not well defined. An annual report is mandated. It is to be submitted to the IRB, which traditionally does not collect patient records on procedures or research. More detailed data could allow researchers to review procedures, determine the efficacies of treatments, and recommend treatment protocols. The creation of a public registry with these data and a set of rigorous reporting requirements could be a good resource for further understanding the impact of SCBI, a proposal several scientists in the state have made.

Several lawmakers agreed with this proposal, including Sen. Bettencourt and Rep. Parker. ''We want to see [a registry] with structure that documents it, that there's data through the IRBs that's rolled up, that there's good oversight from the Texas Medical Board, and importantly, that the success of the treatments gets known,'' said Sen. Bettencourt [10]. In addition Rep. Parker remarked, ''I'm hopeful, obviously, always and will continue to support greater transparency, these kinds of registry concepts and so forth.'' Rep. John Zerwas agreed, ''I personally, I am a big fan of registry … I think that it's a great resource of knowledge and ability to prove things that happen.'' Since the state registry for SCBIs was not part of the original legislation, the state might have to pass a new bill that would create the registry as well as describe report requirements.

Furthermore, not only is data limited on the safety and efficacy of SCBI, but scientific research is also moving forward, which might make these SCBIs obsolete. For instance, scientists are working on advancing our ability to augment the potential benefits of adult stem cells already residing in the human body instead of transplanting autologous stem cells. This work is focused on utilizing small molecules or epigenetic approaches combined with engineering strategies to promote cellular regeneration. These techniques could potentially limit side effects and be more effective than transplanted SCBIs.

Finally, safety concerns have been raised since the treatments are unproven as well as concerns that this new law will open up the state to unsavory and predatory practices by individuals preying on vulnerable patients. Furthermore, without FDA oversight, there is no guarantee that clinics offering experimental SCBIs will follow through with rigorous outcome testing and treatment of unintended side effects, further risking patient safety.

Conclusion

HB 810 is Texas' first step at an expansion of the Right to Try law. While the measure is extremely controversial, there are hints by legislators that this might not be the end. Sen.

Bettencourt stated publically, ''We're trying to use a generic tool that people understand as adult stem cells and try to open up the permutations to let—to try as many therapies we can in a logically controlled manner.'' In addition, Rep. Parker mentioned he believed ''support from [these SCBI patients will] be able to actually move forward to this gateway … to explore adult stem cell for whether or not you are terminally ill or chronically ill'' [10].

While HB 810 opens up access to patients, it also increases significant risks for their safety and financial cost for something that might have no positive impact on their disease. Truly understanding the impact of SCBIs requires scientific rigor, and accurate outcome data reporting must be pursued to ensure the safety and efficacy behind such procedures. This information must be readily available so that patients can make informed decisions before electing to pursue such treatments. The creation of the SCBI registry could allow for some level of scientific rigor, provide a centralized data source, and offer the potential for better informed patient choices, and might be the best option for the state to help protect patients.

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Author Disclosure Statement

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References

1. Lau D, U Ogbogu, B Taylor, T Stafinski, D Menon and T Caulfield. (2008). Stem cell clinics online: the direct-to-consumer portrayal of stem cell medicine. Cell Stem Cell 3: 591–594.
2. Ogbogu U, C Rachul and T Caulfield. (2013). Reassessing direct-to-consumer portrayals of unproven stem cell therapies: is it getting better? Regen Med 8:361–369.
3. Matthews KRW and AS Iltis. (2017). Unproven stem cell-based interventions: advancing policy through stakeholder collaboration. Tex Heart Inst J 44:171–173.
4. Turner L. (2015). US stem cell clinics, patient safety, and the FDA. Trends Mol Med 21:271–273.
5. Turner L and PS Knoepfler. (2016). Selling stem cells in the USA: assessing the direct-to-consumer industry. Cell Stem Cell 19:154–157.
6. U.S. Department of Health and Human Services and Food and Drug Administration. (2017). Regulatory considerations for human cells, tissues, and cellular and tissue-based products: minimal manipulation and homologous use. www.fda.gov/downloads/biologicsbloodvaccines/guidancecomplianceregulatoryinformation/guidances/cellularandgenetherapy/ucm585403.pdf. Assessed June 25, 2018.
7. TX Legislature. (2017). HB 810: relating to the provision of certain investigational stem cell treatments to patients with certain severe chronic diseases or terminal illnesses and regulating the possession, use, and transfer of adult stem cells; creating a criminal offense. https://legiscan.com/TX/text/HB810/2017. Assessed June 25, 2018.
8. Rubin MJ and KRW Matthews. (2016). The impact of right to try laws on medical access in the United States. Baker Institute Policy Report, No. 66.
9. Johnson R. (2018). Letter to FDA: agency should comply with right to try law. https://www.hsgac.senate.gov/media/majority-media/johnson-to-fda-agency-should-comply-with-right-to-try-law Accessed September 4, 2018.
10. Baker Institute. (2017). Charlie's law: increased access to stem cell interventions in Texas. www.bakerinstitute.org/events/1883/. Assessed June 25, 2018.

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