In Support of S. 2912

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Hearing: Exploring a Right to Try for Terminally Ill Patients

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I would like to thank the committee for this opportunity to testify in support of S 2912, known as the Trickett Wendler Right to Try Act. As President & CEO of a biopharmaceutical company developing treatments for currently incurable diseases; as a member of the advisory board that helped craft the model right to try act which has been making it's way through the states; and as the father of a son diagnosed with a grapefruit sized brain tumor at age 4; I have been involved in the scientific, FDA regulatory, business, legislative and patient advocacy arenas germane to this issue for over two decades. S. 2912 is a good bill that will provide hope and comfort to many patients diagnosed with fatal diseases; and it will accelerate the effort to find cures for currently incurable diseases.

It is not without its controversies and issues, but I believe many of the criticisms of the bill are the result of misinformation and lack of understanding of how the bill actually will work. I would like to spend my limited time here today setting the record straight and answering these criticisms.

An often heard criticism is that the bill allows unsafe medicines to be foisted on an unsuspecting public. Nothing could be further from the truth. This bill, like **all** of the state passed bills that have preceded it, relies heavily on the proven safety track record of the FDA. No treatment may be administered under this bill unless it has already successfully passed through an FDA safety trial; AND is continuing in the FDA approval process into later stage trials. This insures that there is a continuing evaluation of the safety of any treatment administered under a right to try act. If a company, for any reason, safety or otherwise pulls a drug from the FDA que it is no longer allowed to be administered under the act. I would note that this is not a protection afforded to the public with respect to approved drugs.

I will also point out here that treatments for fatal diseases are obviously extremely difficult to develop. The failure rate is obviously extremely high and so no company enters into such an undertaking lightly. Even in small biotech companies such as Neuralstem, which I had the honor to help found and run for 15 years, we spent tens of millions of dollars over a decade to get our ALS product into the clinic. The body of science required, and sophistication and volume of pre clinical safety data required to simply get into an FDA approved trial is an enormous undertaking. The point is, in addition to a successful phase

one safety trial, there is a large body of pre clinical safety data and manufacturing process purity data behind all of these treatments, by definition; no exceptions. I would also point out that the FDA often continues to request continuing non human safety testing as a drug moves through the approval process and new data becomes available. These are heavily vetted and continually investigated treatments being made available by the act.

I would also like to debunk the myth that this is somehow an "anti FDA" bill. Again, nothing could be further from the truth. As just explained, the bill's heart is the safety net that continuing FDA oversight provides. An oversight by the way, that is universally acknowledged as the gold standard for the world. Even proponents of the Right to Try movement such as the Goldwater Institute, who might believe that the right to try is based on a Constitutional principle, and might disagree with the idea of Federal pre emption have insisted on this FDA oversight principle on safety. This is not an anti FDA effort.

I would like to address the argument I have heard that this act gives false hope to patients, promising cures. As I mentioned I have been involved in the ALS and brain tumor patient community for a long time; as well as patient and caregiver communities in other currently incurable but not fatal diseases. The biotech and pharmaceutical industry is meticulous about educating patients as to the experimental nature of their treatments in the trial stages. While we are always hopeful that we are on to something substantial, patients are always informed that they are helping to accelerate research, and not being promised a cure. I will also tell you that these diseases are devastating emotionally to patients and their caregivers. They often feel a sense of hopelessness and despair. In ALS, some make their peace with their fate and focus on their remaining time. But many tell you they would like to "go down fighting" and being part of experimental research, whether in a clinical trial setting or through a right to try compassionate use setting, lends an additional sense of purpose to their lives. Having hope for many, is an essential element to improving their quality of life, even if it is a small hope. Patients and caregivers alike know that even every failure brings us closer to an actual cure and their doctors will tell you that that can add great comfort and some sense of control to their lives as they struggle through their illness. The fact that a doctor must administer these treatments ensures that patients have this information before undertaking any treatment. So yes, this is a choice, but it is an informed choice. This is a right to try act, not a right to cure act and all patients will understand this as a result of the mechanisms built into this bill.

I would like to address two areas more related to the “business side” of Right to Try. Yes, companies need to be able to charge for the treatment, and they should be free to set the cost. Almost all new experimental treatments for fatal diseases are going to be modern medicines; cell and/or gene therapies, monoclonal antibodies etc. The ability to apply resources to provide treatments while simultaneously conducting clinical trials will vary from company to company. There can be no “one size fits all” cost formula that would be either fare or productive. Only a company knows its true opportunity costs in such a situation. Indeed, there is nothing in any of the state bills, and of course nothing in this federal bill, that requires a company to make its treatment available under right to try. This too must be decided by the company itself.

I have heard the criticism that actually carrying this out is simply “too hard”. That all of the unresolved issues around insurance reimbursement and liability, both professional and product, added to a fear of “rationing” because of scant availability and a host of others make this unworkable. It is true that all of these issues must be addressed, but it also true that we have these exact same issues with respect to approved drugs; and that in fact we are an industry completely based upon the proposition that all of these issues are addressable. There are people in the applicable chairs throughout the space who work on these issues all day every day with respect to approved drugs. This will be no different. Not every resolution will be perfect, and not everyone will be happy with how these issues are resolved, but that is also true with approved drugs; and just as with approved drugs, all of these issues will get resolved.

Finally, I have heard it said that there are already dozens of states that have passed these laws and yet no one is being treated. I can tell you wearing my industry hat that no company will feel comfortable acting under these state laws, until the protections afforded companies under this Federal law are enacted. This Bill is essential to unlocking the promise of all of the Right to Try acts.