Statement of Matthew Bellina

HSGAC Hearing – September 22, 2016

As a 32 year old father, U.S. Veteran, and terminally ill ALS patient, I wanted to clear up some misconceptions about S. 2912 and HR 3012—the Right to Try Acts

I should begin by clarifying a key point. No one who supports the federal Right to Try Acts or the state laws they protect wants to undermine the FDA or relax the standards that must be met for drug to be officially approved by the FDA. We simply believe that we can do better at getting promising treatments to sick and dying Americans.

A recent GAO report found that the pace of scientific discovery is stifled by an overly burdensome regulatory environment in the FDA's clinical trial protocol. ALS is a perfect illustration. There are 37 known ALS genetic mutations that make up less than 10% of all cases. The other 90% of ALS patients are suffering from a similar disease (or diseases) of unspecified cause. Under the current regulatory environment, all ALS patients are put into the same clinical trial cohorts in the hopes that one drug might show efficacy in the overall patient population. This is bad science and can never be successful.

Right to Try laws will let doctors look at individual patient biomarkers and work with pharmaceutical companies to use known compounds that target a patient's specific disease profile. This is the future of medicine—and we have the technology, just not the regulations, to allow us to do this now. I cannot speak directly for them, but I surmise this is why the ALS Association recently endorsed S. 2912.

I have heard the argument that this legislation would subject patients to risk and they would have no legal recourse if things go wrong. I have heard the only thing worse than a terminal illness is being terminally ill and suffering a major complication as a guinea pig in an experimental treatment that you had to pay for.

Without any intended insult, I do not believe it is the role of interest groups or bioethicists who have never met me to dictate how I should find value in my remaining days. If some believe that living without hope is superior to living with the risk of side effects that is their personal business.

My motor neurons are dying and without treatment I will suffocate under the weight of my own chest. I am willing to make informed choices with my doctor, based on the individual nature of my disease. Furthermore, any compound that would qualify as a Right to Try drug would already have passed safety trials and be in an active FDA-approved Phase 2 or Phase 3 trial. In other words, the FDA has already deemed the compound worth the risk of further exploration.

Why shouldn't I be given the same right as the limited number of patients lucky enough to get into clinical trials?

I have also heard the argument that the FDA's current compassionate use program already provides patients with the opportunity to try investigational drugs. But this program is severely flawed. The

FDA itself has acknowledged these flaws by attempting to streamline the application process and proposing a new office within the agency to help dying people navigate its bureaucracy.

As long as bureaucrats are making the decisions about which terminal patients are privileged enough to have access to investigational treatments, the academic and scientific integrity of biopharmaceutical research will be inhibited, in the best-case scenario. In the worst-case scenario, we will continue to repeat the same sample bias, which has failed to cure heterogeneous diseases for over 60 years.

I have heard the argument that bypassing the oversight of the FDA is not in the best interest of patients or public health. This is a straw man argument. No one is asking to bypass FDA oversight. Lawmakers must realize that only drugs active in Phase 2 or Phase 3 trials will qualify as Right to Try drugs. The FDA will still have control over what drugs can be bought and sold in the United States. And because the availability of drugs under Right to Try depends on the FDA trail process, the “Gold Standard” remains completely intact.

The greatest weakness of these bills is that many pharmaceutical companies may choose not to participate. If that happens then we have missed a great opportunity, but no one will be overtly harmed. On the other hand, the risk of not passing these bills is that a good drug may languish for 10-14 years in the FDA pipeline and countless Americans will die. Their lives will end without the chance to exercise their Constitutional freedom to make choices regarding their life, liberty, and their own pursuit of happiness.