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Everyone Deserves the Right to Try: Empowering the Terminally Ill to Take Control of their Treatment

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EXECUTIVE SUMMARY

In 2002, Kianna Karnes, a 41-year-old mother of four children, was diagnosed with kidney cancer.[1] She was treated with interleukin-2, the only medication approved by the Food and Drug Administration (FDA) at the time to treat her disease. When that treatment failed, her father began researching investigational medications, learning in 2004 that both Pfizer and Bayer were conducting clinical trials for new investigational medications to treat kidney cancer. Karnes was ineligible for the clinical trial because her cancer had previously spread to her brain. Although her brain tumors had been removed, she was still disqualified from joining the clinical trial, so her father sought expanded access for his daughter. Months passed before he was able to secure access for his daughter. He contacted Congressman Dan Burton's (R-IN) office for assistance, and drew media coverage of Karnes' struggle in the *Wall Street Journal*. On March 24, 2005, the FDA notified the family that it had approved a single-patient IND for Karnes. Tragically, it was too late—Kianna Karnes died the same day access was approved.[2] Less than a year later, both drugs were given final FDA approval to treat advanced kidney cancer. Speaking after his daughter's death, her father said, "I don't know that either of these drugs would have saved Kianna's life, but wouldn't it be nice to give her a chance?"[3]

In the case of Kianna Karnes, she had a better chance than most patients at receiving expanded access. As her father explained, "Here is a case where her old man understood clinical trials. I knew about compassionate use; I had a friendship with a powerful member of Congress; I've got the *Wall Street Journal* behind me. But I still couldn't save her life. Now, what about the thousands of people out there who don't have these kinds of resources available to them?"[4] To most patients, and many physicians outside of major institutions, the process of obtaining expanded access is excessively time-consuming and extremely difficult to navigate.

For patients suffering from terminal illnesses, the FDA is the arbiter of life and death. These patients, suffering from diseases ranging from ALS to Zellweger Syndrome, face little chance of recovery. For patients like Kianna, investigational medicines provide a glimmer of hope. The FDA, however, often stands between the patients and the treatments that may alleviate their symptoms or provide a cure. To access these treatments, patients must either go through a lengthy FDA exemption process or wait for the treatments to receive FDA approval, which can take a decade or more and cost hundreds of millions of dollars. Sadly, over half a million cancer patients and thousands of patients with other terminal illnesses die each year as the bureaucratic wheels at the FDA slowly turn.[5]

Patients should be free to exercise a basic freedom – attempting to preserve one's own life. The burdens imposed on a terminal patient who fights to save his or her own life are a violation of personal liberty. Such people should have the option of accessing investigational drugs which have passed basic safety tests, provided there is a doctor's recommendation, informed consent, and the willingness of the manufacturer of the medication to make such drugs available.

States should enact "Right to Try" measures to protect the fundamental right of people to try to save their own lives. Designed by the Goldwater Institute, this initiative would allow terminal patients access to investigational drugs that have completed basic safety testing, thereby dramatically reducing paperwork, wait times and bureaucracy, and, most importantly, potentially saving lives.

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patient who met the stated criteria from accessing investigational medications. Likewise, other procedural burdens such as the IND application and IRB review requirement could be deemed undue burdens and either eliminated or drastically curtailed.

The concept of ordered liberty cannot include allowing a government agency to promulgate and enforce regulations that impair an individual's health or cause death by denying or delaying access to potentially life-saving medications. The way in which the FDA currently regulates access to investigational medications may be rational for non-terminal patients, but its application to terminal patients, who lack other treatment options, is not. Preventing such a patient from accessing a potentially life-saving medication will, without question, result in the fulfillment of the diagnosis — death.

Without the action of state lawmakers, terminal patients are at the mercy of a federal bureaucracy that can literally cause death by delays, denials, and unnecessary procedural requirements.

It has long been established that the U.S. Constitution creates a floor of protection for individual rights – not a ceiling. States can and do provide additional and enhanced protections for individuals.

Conclusion

From her sickbed, Edie Bacon wrote of the travails a terminal patient faces and made a final plea for the only medication that might save her. "The government wants proof of efficacy before it will allow me to take this drug outside of an approved trial. But the 'proof' is years away, and I need the drug now. It's safe. It might work. Johnson & Johnson would let me have it if they could do so without the threat of a government hassle. But they're so caught up in the FDA web that the life of an individual patient has no importance whatsoever. Without ET 743, I'm a dead woman walking. Five kids are going to wonder why they're left without a mother. Won't somebody help me get this drug?"[130] Edie died two years later, but there are thousands of patients who face this same battle every day – patients who have to make the same pleas that Edie did for a chance to try to protect their own lives.

Such pleas should anger anyone who believes in the concept of personal liberty. No free person should have to come to the government as a supplicant to beg for a right to try to save his or her own life. In a country dedicated to the idea that all people have certain "unalienable Rights, that among these are Life, Liberty, and the Pursuit of Happiness," no government official should have the power to deny a person's last chance at all three – life, liberty, and happiness.[131] Yet that is the power the FDA wields today. States should challenge this regulatory authority by passing Right to Try and returning medical decision making back to the rightful hands of patients and doctors.

The Goldwater Institute

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