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Right to Try: Patient Advocacy for End of Life Drug Access

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When it comes to cutting-edge therapies in medicine, we have heard the hope-inspiring story before: a terminally ill patient enrolls in a clinical trial and, against all odds, overcomes the disease. In 2011, Jenn McNary, mother of two young boys with Duchenne Muscular Dystrophy (DMD), embarked on such a story. Upon diagnosis of both her children with DMD, she was told that fatality was 100%. In 2011, she welcomed the hope that came with a clinical trial for a drug called Eteplirsen.[1] Only the younger child, however, qualified for the clinical trial. As the clinical trial progressed, marked improvements in muscle development were apparent in her younger child Max. In sharp contrast, her older son Austin continued to deteriorate due to DMD; eventually he was unable to move his body. After three painful years of watching her older son slowly deteriorate, the Food and Drug Administration (FDA) finally permitted a clinical trial that Austin could join in 2014.[2] Clinical trials offer a way for patients to access investigational therapies. Many of the sickest, however, are often disqualified from participating. An estimated 97% of patients are unable to access or qualify for clinical trials.[3] These patients have few options of resort.

The US is known to have one of the strictest drug approval and regulatory policies in the world. On average, it takes 12 years for an experimental drug to go from the laboratory bench to the pharmacy shelf.[4] The rigor and standard of the FDA comes with historical precedents that have revealed the widespread harm that can result from improper assessment and evaluation.[5] For the terminally ill, however, the potential benefit of a treatment outweighs the "risk" of inevitable death. The 12 years that it might take for a drug to possibly be approved by the FDA represents time precious to patients fighting for their lives.

In the area of improved drug access, patient advocacy has played an influential role. In response to the demands of AIDS patients for access to investigational drugs in the 1980s, the FDA initiated its Expanded Access Programs to allow limited access of investigational drugs outside the clinical-trial setting.[6] The number of approved Expanded Access Programs, however, has been minimal relative to patients' needs. For example, as of 2013 there were 60,000 ongoing clinical trials, but only 210 ongoing expanded access trials, less than 1 percent. And, in 2010, there were 1,014 patients approved for new investigational drug access across all diseases while there were 1,529,560 new cases of cancer alone in the same year. As can be seen, there is a clear need for improvement in the area of drug access for terminally ill patients.

The FDA's Expanded Access Program places a constraining conflict of interest against the needs of the patient. From the patient's perspective, there is nothing to lose and everything to gain from a promising experimental drug therapy. From the perspective of the FDA, it must protect the public from ineffective therapies. While these two perspectives are not mutually exclusive, the FDA remains aware that it places its reputation and authority at risk with each newly approved therapy. As former FDA Commissioner Alexander Schmidt put it, "In all our FDA history, we are unable to find a single instance where a Congressional committee investigated the failure of the FDA to approve a new drug. But the times when hearings have been held to criticize our approval of a new drug have been so frequent that we have not been able to count them."[7] More testing over a longer time decreases the FDA's risk for scrutiny.

While the FDA must protect the public from ineffective therapies, its approach is flawed in the context of terminally ill patients. The FDA has noted that, "For a person with a serious or life-threatening disease, who lacks a satisfactory therapy, a promising, but not yet fully evaluated product may represent the best available choice."[8] Despite understanding a patient's desperation for life, the FDA does not always support the decision of the individual and his/her doctor.[9] In fact, the balance of power is completely sided with the FDA. The FDA is granted expansive veto power even in the face of support by both the will of the patient and the professional recommendation of a doctor. In simple terms, a patient's chance of accessing life-supporting therapy -with the recommendation of medical counsel- can be denied by a bureaucratic veto from the FDA.

The sustainability of such an imbalance of power is untenable in light of patient advocacy for the terminally ill. "The Right to Try" legislation designed by the Goldwater Institute addresses many of the present concerns in order to rectify terminal patients' rights. With The Right to Try legislation, patients are able to circumvent governmental regulation when the following conditions are met:

1. The patient has been diagnosed with a terminal disease.
2. The patient has considered all available treatment options.
3. The patient's doctor has recommended that the investigational drug, device, or biological product represents the patient's best chance at survival.
4. The patient or the patient's guardian has provided informed consent.
5. The sponsoring company chooses to make the investigational drug available to patients outside the clinical trial.

The central tenets of The Right to Try are rooted in the concept of personal liberty. With full bipartisan support, the legislation has been passed in 30 states, most

recently in West Virginia.[10] This legislation acts to protect the interests of the patient and uphold the patient's constitutional rights to medical autonomy. While the FDA's drug approval and regulatory processes may hold a rational framework in the context of non-terminal patients, it directly opposes precedent Supreme Court cases that confirm an individual's, "right to care for one's health and person."[11] The right to try, after all, should be in the hands of the dying individual, should it not?

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