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OPINION

How the Senate Can Help Ted Kennedy

By STEVEN WALKER and RONALD TROWBRIDGE (*Abigail Alliance*)

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The recent news that Sen. Ted Kennedy has brain cancer sharply focuses national attention on the tragedy of all forms of cancer. The senator has a malignant glioma so difficult to treat that half of those diagnosed with it die within a year, and nearly all are dead within two years.

There are many promising new cancer treatments in the pipeline, but under current Food and Drug Administration (FDA) regulations, almost no one gains access to them, no matter how dire the need or how compelling the evidence that the drugs work.



Sen. Ted Kennedy

AP

Most people receiving a terminal cancer diagnosis die before the most promising treatments in the pipeline reach them. Why? Because those tragic events occur on the wrong side of the magical moment when someone at the FDA puts an approval letter on a fax machine declaring the drug they needed – and never got – is "safe and effective."

Congress now has an opportunity to address this problem thanks to Sen. Sam Brownback (R., Kan.) and Rep. Diane Watson (D., Calif.), who recently introduced the Access, Compassion, Care, and Ethics for Seriously Ill Patients Act. The Access Act, said Mr. Brownback at a press conference introducing the bill, provides that "terminally ill patients whose medical needs are unmet by currently available options would be granted access to promising, investigational treatments."

As Ms. Watson observed at the same press conference, "The activism of the AIDS community in the 1990s expedited the marketing to the general public of promising antiretroviral drugs. Today it is my understanding that many AIDS drugs do not have to go through the controversial and questionable Phase III testing with placebo controls. Sadly, the expedited approval of promising new drugs for cancer patients and patients with other life-threatening diseases does not receive the same attention or expedited approval."

We know from personal experience – having received similar diagnoses for our spouses – what Mr. Kennedy and his family face, and it is our hope that he be given access to any promising treatment that can give him the best chance of extending his life. We support that access even if he gets it only because of who he is, a Kennedy and a U.S. senator. Our national shame is that humane access to effective drugs is not available to all with terminal illnesses.

Among the promising new therapies that should be available to Mr. Kennedy is a vaccine being developed at Duke University. The vaccine trains the body's immune system to kill malignant tumor cells, but to leave healthy cells alone. It is safe and effective, increasing average survival for patients with malignant gliomas like Mr. Kennedy's from 14 months to 33 months, based on the results of clinical trials presented in Chicago at a conference on cancer recently. Although the number of patients treated so far is small, the magnitude of the estimated survival difference and the strength of the underlying science makes it very unlikely that the positive effect is due to chance.

But the vaccine faces additional years of randomized trials in which a few hundred patients will get, or not get, the vaccine before the FDA considers approving it. Given what we know about the vaccine, the ethical problems with such trials are obvious, but the FDA will rigidly demand them.

In the meantime, the thousands who won't get into the trials will die waiting. What will we learn? That the survival advantage is a month or two less, or more, than the 19 months already estimated. That's it.

Mr. Kennedy's situation, identical from a regulatory standpoint to the plight of hundreds of thousands of other Americans, shouts to the heavens the humane necessity of urgent reform in the drug approval process to make it work better for people who have serious and terminal diseases.

The Access Act creates a new approval mechanism called Compassionate Investigational Access (CIA) for patients who can't wait. Patients receiving a CIA drug must suffer from a serious or life-threatening disease, be out of approved options and unable to gain access to a clinical trial, provide informed consent, and allow the collection of clinical data from their experience with the drug so we will all know more about the safety and efficacy of new therapies before they are approved for wider use.

The Access Act also improves the FDA's accelerated approval mechanism that has helped to preserve the lives of HIV/AIDS sufferers, but has never been adequately applied to other serious diseases like cancer.

Before his diagnosis, Mr. Kennedy was working on legislation to reinvigorate the war on cancer. The Access Act belongs in that package. Some of his friends in the Senate, including Democrat Bob Casey and Republican Arlen Specter of Pennsylvania, co-sponsors of the Access Act, will support him.

The unavoidable reality for people with a terminal disease is that good cancer drugs are held up behind a one-size-fits-all regulatory wall. The Access Act offers Congress, and Mr. Kennedy, a way to help break the regulatory logjam for all of us.

Mr. Walker is chief adviser and co-founder, and Mr. Trowbridge is adjunct scholar, of the Abigail Alliance for Better Access to Developmental Drugs.

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The FDA Is Killing Crohn's Patients *Science didn't require that I get a placebo.*

By **GIDEON J. SOFER** (A young patient the Abigail Alliance has been trying to help.)

Right now there are millions of individuals whose lives are directly dependent on the rate at which new drugs come to market. I'm one of them. I'm fighting for my life.

To date, half of my intestine has been removed to manage Crohn's disease. Last year, at age 23, I enrolled in a clinical trial for a treatment that could save my life: an adult stem-cell therapy that helps damaged intestinal tissue regenerate from the relentless inflammation and scarring caused by Crohn's.

The sponsor, Osiris Therapeutics, reported that Crohn's patients in the therapy's Phase II trial all experienced clinical improvement after receiving the cells. A Phase III trial for the treatment is now nearing completion, but Food and Drug Administration (FDA) approval could be years away, despite its FDA "fast track" designation.

In accordance with antiquated FDA policies, the Phase III trial is randomized with three groups of patients, and double-blinded, which means neither the doctors nor patients are told what treatment is being administered. One group received full-strength stem cells, another received half-strength, and a third got a placebo (the proverbial "sugar pill"). It appears I got the placebo.

Foregoing all other treatments, I received the four scheduled infusions, and yet my disease progressed with a vengeance. In a matter of weeks, I became dangerously malnourished. I've since been readmitted to the hospital countless times, as my doctors continue to plead with Osiris for information. But Osiris has refused, citing adherence to FDA protocol.

I am now a lab rat. I have no right to know what happened to me in the study, nor do I have a right to try the promising treatment as my health deteriorates. It doesn't have to be this way.

Under the Fifth Amendment's guarantee that "No person shall be deprived of life, liberty or property without due process of law," a critically ill patient should have access to a potentially lifesaving drug that has been deemed safe for human consumption, if the patient agrees to bear the risks involved. But earlier this year, the Supreme Court refused to hear a case on the issue, denying countless patients their right to pursue life.

Thankfully, some members of Congress have stepped in to ensure our rights as patients. In May, Sen. Sam Brownback (R., Kan.) and Rep. Diane Watson (D., Calif.) introduced the Access, Compassion, Care and Ethics for Seriously Ill Patients Act. If passed, this bipartisan legislation will begin to restore the rights of millions of patients by widening access to promising investigational drugs.

Human clinical research is an intricate scientific and moral process, but it does not justify taking immoral advantage of patients. Tragically, FDA and Osiris think it does.

Typical approval protocols almost always guarantee patients taking the placebo access to the actual drug -- at the very least -- after the study has ended. But in what appears to me a deliberate act of cruelty, Osiris hung its patients out to dry without any recourse, refusing to

confirm which patient got what. The FDA has endorsed Osiris's decision by enabling it to proceed with the study.

Withholding a potential cure is just as bad -- if not worse -- than the potential death sentence of a serious illness. If patients like myself have the audacity to put their lives on the line for the betterment of science and those in their predicament, their decision should not only be embraced, it should be rewarded.

Furthermore, trials without ethical recourse can lead to inadequate and incomplete data, compromising the integrity of the study. If trial patients are treated like lab rats, they won't feel obliged to cooperate unconditionally and report accurate data -- something the FDA and the drug industry rely on heavily, but have failed to consider.

Everyone agrees it is a fundamental right for patients to dictate their course of treatment with FDA-approved drugs. So why do the rules evaporate at the most critical moment, when the only life-preserving options are highly promising investigational drugs?

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