

If FDA blocks meds, how will government health care work?

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"Life in a glass coffin" — that's how one patient described living with Amyotrophic Lateral Sclerosis (ALS), also called Lou Gehrig's disease. The condition indiscriminately afflicts 15 people each day in the U.S., robbing ALS sufferers of the voluntary use of their muscles and leading to death within two to four years.

No cure exists, but one drug treatment has recently offered hope that the disease's progression can be slowed.

But in fighting for this chance at life, patients and their doctors found they first had to fight their own government for access to the drug, known as Iplex. Their story foreshadows everyone's health care future if "reform" strips sick Americans of their freedom to choose treatment options and take informed, appropriate risks.

The Food and Drug Administration (FDA) approved Iplex in 2006 for use in infants and children with growth problems. The following year, doctors and researchers in the U.S. and Europe concluded that Iplex could be effective at combating Lou Gehrig's disease, and some patients began receiving it.

For dozens, Iplex slowed or stopped their muscular degeneration. Notably, no ALS patient experienced side effects or had safety concerns with the drug.

A patent infringement lawsuit involving Iplex's producer forced the drug off the market temporarily in 2007, at which point, FDA became involved. The parties to the legal dispute agreed that ALS patients could receive Iplex, but FDA insisted that patients first navigate the agency's "compassionate use" process.

This process empowers the agency to determine whether terminally ill patients can use an unapproved treatment. FDA officials claim to appreciate the life-or-death nature of this authority, asserting in a legal brief filed with the U.S. Supreme Court that their "standards for terminally ill patients are accommodating," and that "most — indeed nearly all" requests are approved.

While nearly 100 ALS patients in Italy were once again benefiting from Iplex, desperately ill U.S. patients had to file requests with FDA and wait. Last January, patients and their families across the country were shocked to learn that FDA had denied their petitions. Among other issues FDA cited, it pointed to safety concerns. Outraged advocates wondered how a drug approved for children could possibly be unsafe for adults with excruciating muscle failure. "Safety?" one mother remarked to The New York Times. "What's safe about ALS?"

Last February, Washington Legal Foundation (WLF) appealed to FDA on behalf of some ALS patients, arguing that the agency violated its own regulations when denying the

applications. FDA's safety standards for Iplex were far higher than those it had imposed in other emergency access situations. A lower standard, as FDA itself has acknowledged, is called for with terminal patients, so they can make their own choices based on their condition.

On March 10, FDA provided news that was joyous for some ALS patients, but sobering for others. Those whose formal compassionate use petitions had arrived at FDA by March 6 would receive Iplex. Those who had not filed petitions by this seemingly random date had to enter a lottery to participate in a then-unscheduled Iplex clinical trial.

The cautionary tale, however, doesn't end there. In late April, WLF heard about an ALS patient who had received a letter from FDA rejecting an Iplex compassionate use petition she had mailed on March 6.

In its rejection, FDA cited the same questionable safety concerns it had relied upon when rejecting prior petitions. Also, Iplex's manufacturer informed her that because FDA received her petition after March 6, it was rescinding its earlier agreement to supply her with Iplex.

FDA's audacity in informing one ALS patient on April 21 that Iplex was unsafe, after telling other patients on March 10 they could safely use it, is breathtaking. But far worse is that this public health agency denied a terminally ill woman's plea for a last-chance treatment because she missed an arbitrary deadline — about which she was never informed — by at most three days.

ALS patients and their families deserve an explanation as to why FDA initially felt their plight was unworthy of agency "compassion," and then why it reversed course for some on Iplex, but slammed the door on others. They will probably never get a straight answer, but for the rest of us, this debacle is rather revealing.

If federal officials today can so capriciously deny one treatment option to a handful of terminal patients, Americans certainly can't expect consistent and rational outcomes from a reformed health care system where regulators dictate what is best for government, not patients.

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