



March 10, 2009

IN MAJOR VICTORY FOR WLF, FDA GRANTS ALS PATIENTS ACCESS TO PROMISING NEW DRUG

The Food and Drug Administration (FDA) today announced that it has reversed course and will now grant patients suffering from ALS (Amyotrophic Lateral Sclerosis, or “Lou Gehrig's Disease”) access to Iplex, a promising new experimental drug, on a compassionate use basis. The decision was a victory for the Washington Legal Foundation (WLF), which had filed formal appeals from FDA's January 23, 2009 decision to deny access to Iplex. ALS is a fatal neurodegenerative disease for which there is no known cure. Access to Iplex provides ALS patients some measure of hope; they have no other available treatment options.

In the past several months, several dozen ALS patients and their doctors have filed applications for Treatment INDs (“Investigational New Drugs”) for “compassionate use” access to Iplex, a drug developed and manufactured by Inmed, Inc. Iplex has not been approved by FDA for treatment of ALS, but has been determined by FDA to be safe and effective in treating other medical conditions. Moreover, Iplex is now being administered to ALS patients in Europe on a compassionate use basis – with promising results.

In a series of letters dated January 23, FDA's Division of Neurology rejected the INDs, asserting that there was insufficient evidence of Iplex's safety and efficacy in treating ALS. Acting on behalf of four of the ALS patients, WLF appealed the denial to Acting FDA Commissioner Frank Torti. WLF argued that the denials violated FDA's own regulations, which contemplate that “virtually all” Treatment INDs will be granted to patients with life-threatening illnesses who lack other effective treatment options.

Today's announcement reverses FDA's prior decision. FDA announced: (1) it will approve the Treatment IND applications from all ALS patients who filed by March 6, 2009; (2) all other interested ALS patients can enter a lottery for participation in a clinical trial to be conducted by Inmed. The details of that lottery were not spelled out.

“Today's decision is a significant victory for the ALS community,” said WLF Chief Counsel Richard Samp. “It is a belated recognition by FDA that denying access to developmental drugs under these circumstances serves no public health interest. When critically ill patients lack access to FDA-approved treatment options, it makes no sense for FDA to claim that it is protecting their health by denying them access to promising drugs with

unproven safety records. By denying access under those circumstances, FDA is essentially telling patients to give up all hope and wait passively for death," Samp said.

Although FDA did not explain how it would conduct its lottery for ALS patients who have not yet filed Treatment INDs, the system appears to be designed to ensure that FDA can obtain meaningful data regarding Iplex's safety and effectiveness. It is probable that FDA will direct Inmed to establish a double-blind study, under which all applicants will receive daily injections, but only half of the patients will be receiving Iplex -- the other half will receive a placebo. The study would be double-blinded, meaning that neither the doctor nor the patient would know whether Iplex is being administered.

FDA's announcement stated that the lottery system was being adopted because Iplex is in short supply. While Iplex may indeed be in short supply, that shortage likely has little to do with FDA's decision. If a supply shortage were the only problem, there would be no reason for FDA to get involved; Inmed would be free to work out its own procedures for distributing the available product. Rather, the most plausible reason for the lottery system is to ensure that, by allocating Iplex to only a portion of all applicants, FDA can obtain meaningful data regarding Iplex's safety and effectiveness.

FDA has established a compassionate use program that is intended to provide access to developmental drugs for patients who are terminally ill and have no other effective treatment options. FDA expanded the program in response to a lawsuit filed by WLF in 2003, *Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach*, a constitutional challenge to FDA restrictions on access to drugs under development. After an initial WLF victory in the appeals court, FDA ultimately prevailed by assuring the courts that its restrictions were actually minimal. It told the U.S. Supreme Court that "virtually all" requests for "compassionate use" access to drugs are granted.

While Iplex's efficacy has not been established by any well-controlled clinical studies, there is substantial anecdotal evidence that Iplex has been helpful when administered to ALS patients in Italy. In today's announcement, FDA stated that it had been in communications with Italian officials. FDA stated that its decision to reverse course was prompted in part by assurances from Italian officials that no major safety concerns had arisen among doctors who were administering Iplex to their ALS patients.

WLF is a public interest law and policy center with supporters nationwide. It devotes a significant portion of its resources to protecting individuals rights.

* * *

For further information, contact WLF Chief Counsel Richard Samp, (202) 588-0302. Copies of WLF's appeal briefs are posted on its web site, www.wlf.org.