News Release

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FDA URGED TO GRANT ALS PATIENTS ACCESS TO PROMISING NEW DRUG

The Washington Legal Foundation (WLF) today filed formal appeals with the Food and Drug Administration on behalf of clients who are suffering from a life-threatening disease, urging FDA to grant them access to a promising drug for the treatment of their disease. WLF has pledged to pursue relief in the courts if its appeals are denied.

WLF's clients – Joshua Thompson of Virginia Beach, Virginia and Mark Smith of Bonney Lake, Washington – are suffering from ALS (Amyotrophic Lateral Sclerosis, or "Lou Gehrig's Disease"), a life-threatening neurological disease for which there is no known cure. The drug in question is Iplex, a drug developed and manufactured by Insmed, Inc., a Richmond, Virginia manufacturer of biological products. FDA has denied Thompson and Smith access to Iplex, despite the fact that both Insmed and their personal physicians believe that Iplex might provide them with significant benefits. Iplex has not yet 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.

WLF's appeals from FDA's denial of access to Iplex are directed to Acting FDA Commissioner Frank Torti. The appeals charge that the denial violates FDA's own regulations, which promise liberal access to developmental drugs to patients with life-threatening illnesses and who lack any other effective treatment options. WLF noted that FDA stated repeatedly in court filings that its policy is to grant "virtually all" compassionate use applications that are supported both by the patient's doctor and by the drug manufacturer. WLF charged that FDA's expressed concern – that it cannot be sure that Iplex will not endanger the health of ALS patients – ring hollow, when one considers that in the absence of treatment, all ALS patients face the prospect of near-term death.

"FDA's refusal to grant access to Iplex serves no public health interests and is a virtual death sentence for ALS patients," said WLF Chief Counsel Richard Samp after filing the appeals. "Both Joshua and Mark were diagnosed with ALS two years ago, and their health is deteriorating rapidly. FDA has suggested that ALS patients should try to enroll in on-going clinical trials, but such trials are not open to those such as Joshua and Mark whose illnesses are more advanced," Samp said.

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 available to them. FDA expanded the program in response to a lawsuit filed by WLF several years ago, *Abigail Alliance for Better Access to Developmental Drugs v. von Eschenbach*, a constitutional challenge to FDA restrictions on access to drugs that are still under development. After an initial WLF victory in the appeals court, FDA ultimately prevailed by assuring the courts that its restrictions were actually quite minimal. It told the U.S. Supreme Court that "virtually all" requests for "compassionate use" access to drugs are granted.

Despite those assurances, FDA has denied more than a dozen applications for access to Iplex, filed by ALS patients and their doctors. The applications took the form of requests for what are known as "Treatment INDs" (an acronym for "investigational new drugs"). FDA regulations provide that Treatment INDs should be granted liberally to terminally ill patients. FDA nonetheless denied the Treatment INDs on the ground that not enough was known about the safety and efficacy of Iplex. WLF's appeal responded that safety should not be an issue because: (1) ALS patients are going to die in the near term without treatment, so they should be permitted to assume any minimal increased safety risk; and (2) Iplex has already been shown to be sufficiently safe for use in healthy patients that FDA has approved it to treat children with short stature. WLF noted that ALS patients would have been able to get access to Iplex on an off-label basis but for a patent dispute that has temporarily halted all prescription sales of Iplex.

WLF also argued that while Iplex's efficacy has not been definitely established by any well-controlled clinical studies, there is substantial anecdotal evidence that Iplex has been quite helpful when administered to ALS patients in Europe. WLF charged that the real reason that FDA is blocking the Treatment INDs is its desire to pursuade Iplex's manufacturer to launch an expensive clinical study of Iplex's effectiveness in treating ALS. WLF argued that any such study could never begin soon enough to help WLF's clients, and that there is no money at present for such a study. That lack of funding is a recurring problem for diseases (such as ALS) with a relatively small patient population.

WLF has other clients for whom it will be filing similar appeals in the near future. FDA has pledged to act on the appeals within 30 days.

WLF is a public interest law and policy center with supporters in all 50 States. WLF devotes a significant portion of its resources to protecting the constitutional and civil rights of individuals.

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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.