# FDA panel considers 1st drug for chronic fatigue syndrome

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Only some patients got relief from the experimental treatment, experts say.

(HealthDay)—A U.S. Food and Drug Administration advisory committee will meet Thursday to consider approval of the first drug to treat chronic fatigue syndrome.

The experts will discuss the risks and benefits associated with the intravenous drug rintatolimod (proposed brand name Ampligen). The drug's maker, Hemispherx Biopharma of Philadelphia, failed to win the FDA's OK in 2009 because of concerns about study methodology.

Experts said they would welcome a treatment for this disabling condition that affects as many as 4 million Americans, mostly women. There is no cure, but the drug appears to reduce symptoms for some patients.

"It does seem to help at least a subset of patients significantly. For others, there isn't a significant response," said K. Kimberly McCleary, president of the Chronic Fatigue and Immune Dysfunction Syndrome Association of America.

"This drug has been studied in chronic fatigue syndrome since the late 1980s, so it's been around for a while," McCleary added.

Dr. Nancy Klimas, professor of medicine at Nova Southeastern University College of Osteopathic Medicine in Fort Lauderdale, Fla., who is part of an ongoing trial of the drug, said some of her patients have benefitted from the drug. Now there needs to be a way to identify which patients will do well on the drug, she added.

Chronic fatigue syndrome is little understood, and Ampligen's approval would give patients some standing with their insurers, Klimas said.

"Even a single approved therapy, even if it were one I choose not to use, would be very helpful when I am arguing with insurance companies to legitimize the condition and that it is serious enough to require an intervention," Klimas said.

According to the drug company, Ampligen is a new type of drug called a nucleic acid compound, which uses specially made RNA to target a variety of diseases. Hemispherx believes the drug has the potential to fight HIV, kidney cancer and melanoma in addition to chronic fatigue syndrome.

The drug is said to work by modulating the immune and antiviral functions in diseased cells.

One drawback of the treatment is that it needs to be infused twice a week, Klimas said. It also is very expensive, she said.

The maker of the drug couldn't estimate the retail cost but said the manufacturing cost is about $1,000 a month per patient.

The FDA denied approval for Ampligen in November 2009 because of concerns about the way two studies were conducted—too few patients, a protocol change and an early end to one study. This time around, the FDA will review a new analysis of one trial result submitted in 2009, but not a new study.

It's possible that a lack of supporting data could again hold up approval. "Multiple conduct issues with the trial suggest that results should be interpreted with caution," the agency said in a letter to the committee. "The confirmatory trial failed to replicate" the results of the first trial.

In September, the FDA conducted a teleconference with people with chronic fatigue syndrome who had used the drug. These anecdotal reports were largely positive. What weight, if any, these patient experiences will have on the drug's approval isn't known.

Some experts think chronic fatigue syndrome is a virus; others believe it is a bacteria. It can begin after an illness from which a patient doesn't quite recover, or the symptoms can appear almost overnight, McCleary said.

Symptoms often include flu-like weakness. The one common thread is the inability to do almost anything without becoming totally exhausted, McCleary said. Even simple tasks like reading a magazine can set off a cascade of symptoms that last for days or weeks, she said.

"It's a bone-crushing exhaustion," McCleary said. "There is pain in the muscles and joints that can move from one body part to another, sore throat, headaches like migraines, and trouble falling asleep or staying asleep."

There are mental symptoms as well, including difficulty processing information and a "big problem" with short-term memory, McCleary noted.

The FDA's decision is expected early next year. The agency isn't bound to follow the recommendations of its advisory panels but it usually does so.

**More information:** For more information on chronic fatigue syndrome, visit the U.S. National Library of Medicine.