Researchers welcome new multiple sclerosis drug

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The Food and Drug Administration has approved the drug fampridine-SR for the treatment of multiple sclerosis. Researchers at the University of Rochester Medical Center (URMC) have been evaluating the effects of the drug in MS for more than 10 years- it is the first medication shown to enhance some neurological functions in people with the disease - and their efforts helped pave the way for today's action by the FDA.

"This is a good day for people who suffer from multiple sclerosis," said Andrew Goodman, M.D., chief of the URMC Multiple Sclerosis Center. "Physicians will now have a new tool at their disposal that complements existing disease modifying therapies. For some patients, this drug will be a way to improve mobility and help regain some independence in their daily lives."

Researchers at URMC helped develop the study protocols and lead the clinical trials that demonstrated consistently improved mobility - timed walking speed- in more than a third of patients with multiple sclerosis. This is the first instance in which a drug for multiple sclerosis was found to improve function lost as a result of the disease. Goodman and his colleagues published the results of a Phase 3 clinical trial of the drug in the journal *Lancet* last February.

Fampridine-SR is being developed by Acorda Therapeutics and marketed under the name Ampyra. Goodman has been a consultant and advisor to Acorda for its fampridine studies in multiple sclerosis. The company submitted a new drug application to the FDA in February

2009. An expert advisory panel recommended approval of the drug in October and that recommendation was adopted by the FDA today.

Multiple sclerosis is a disease of the central nervous system and is the most common cause of neurological disability in young adults with an estimated 2.5 million people worldwide suffering from the disease. Some of the most common symptoms are gait difficulties, muscle weakness, numbness or tingling in arms and legs, difficulty with coordination and balance, blurred vision, and slurred speech. Early in the course of the disease the symptoms manifest themselves in cycles of relapse and remission but over time the symptoms of the disease tend to become more permanent and debilitating.

These symptoms are triggered by attacks on cells in the central nervous system by the body's own immune system. Specifically, these attacks damage cells called myelin which form the "insulation" around nerve fibers. When this insulation is damaged, communication in the central nervous system is delayed, disrupted, and even blocked. It is believed that fampridine improves the transmission of signals in the central nervous system of some multiple sclerosis patients by blocking potassium ion channels in nerve cells and restoring signal conduction.

Goodman and his colleague Steven Schwid, M.D., who passed away in November 2008, have been evaluating fampridine for multiple sclerosis since the mid 1990s.

Progress began to accelerate when the license to develop the drug was acquired by Acorda Therapeutics. The primary obstacle then was that the process of evaluating the drug's impact required researchers to devise a new way to think about clinical trial methodology. Most multiple sclerosis drugs are measured by their ability to prevent relapses or slow the progression of the disease, not reverse symptoms once they became established. Goodman and Schwid developed methods for assessing

functional outcomes in multiple sclerosis patients such as measuring walking speed over a 25 foot distance.

In the *Lancet* study, they reported that 35% of patients taking the drug were responders who consistently improved their walking speed by an average of about 25%. While walking was the primary measure, patients also reported that they could walk farther distances, climb stairs better, and stay on their feet longer. In prior research, they also found that there is a strong correlation between the speed patients can walk and the overall measurement of disability resulting from multiple sclerosis.

As for any medication, the clinical trials identified potential side effects and risks. Although the most concerning risk has been for the complication of seizures, so far the incidence of seizure-related events in the ongoing clinical safety studies has remained relatively low at the dose approved by the FDA.

Provided by University of Rochester Medical Center