Thalidomide relieves disabling cough for people with deadly lung disease, study shows

September 17 2012

In the first clinical trial to demonstrate an effective treatment for constant, disabling cough among people with idiopathic pulmonary fibrosis (IPF), researchers at the Johns Hopkins University School of Medicine found that taking thalidomide significantly reduced the cough and improved quality of life.

Results of their study are scheduled to be published in the Annals of Internal Medicine on Sept. 18 in an article titled "Thalidomide for the Treatment of Cough in Idiopathic Pulmonary Fibrosis."

IPF is a progressive, fatal disorder that causes the lungs to become stiff and scarred, preventing oxygen from leaving the lungs to go to the rest of the body. The cause is unknown. Up to 80 percent of people with IPF have a dry, nagging cough, for which no effective treatment is available.

Thalidomide is a potent anti-inflammatory drug that was used to treat morning sickness and aid sleep in the 1950s. It was taken off the market in 1961 after it was shown to cause severe birth defects when women took the drug during pregnancy. Today, thalidomide is prescribed with strict controls to treat several diseases, including multiple myeloma and kidney cancer. It had not been studied for people with lung disease before.

"We performed a randomized, double-blind, placebo-controlled trial of thalidomide in patients with IPF to determine its effectiveness in suppressing cough," says lead author Maureen R. Horton, M.D., a

pulmonary disease specialist and associate professor of medicine and environmental health sciences at the Johns Hopkins University School of Medicine.

"We found that low-dose thalidomide significantly reduced the cough and also improved the patients' quality of life, as demonstrated on established questionnaires known as the Cough Quality of Life Questionnaire and the St. George's Respiratory Questionnaire," says Horton.

For the study, patients either took low-dose thalidomide pills or a placebo for three months. Then there was a two-week "wash out" period in which the patients took nothing, followed by another three months when those who had taken the thalidomide went on the placebo and those who had been given the placebo started taking thalidomide.

Neither the patients nor the researchers knew which group the individuals were in.

Twenty patients completed both arms of the study, 15 men and five women. All were over age 50 and the mean age was 67.

Horton says patients often noticed the difference within two weeks of taking the thalidomide. When they stopped the drug, their cough came back. "At the end of the study, all of the participants said they wanted to continue taking the medicine because their cough had improved."

On average, the patients reported that the frequency of their coughing decreased about 63 percent while they were taking thalidomide, and their respiratory-specific quality of life, such as the ability to do daily activities, improved about 20 percent. They also reported that the aspects of their life impacted by their cough also improved while they were on the drug.

"The constant cough caused by the disease can affect the quality of life in many ways," says Horton. "Some no longer go to church or to social gatherings because people think they are infectious. Other patients may have more pronounced urinary incontinence due to the cough, for example, so it has wide-ranging effects."

Side effects, such as constipation, dizziness and malaise were reported by 74 percent of the participants while they were taking thalidomide, and by 22 percent of those who were on a placebo.

Horton says the idea of testing thalidomide for cough among IPF patients came about because the drug is known to have a powerful effect on decreasing inflammation. Horton and her colleagues conducted a previous smaller study to see if thalidomide would help treat the IPF disease itself, and while the results of that research were not conclusive, they noticed that the patients had significant cough relief.

About 80,000 people in the United States have IPF, although it may be underdiagnosed. The risk increases with age. Treatments for the symptoms include oxygen therapy and pulmonary rehabilitation, but there is no effective treatment for the constant cough, which is a hallmark of IPF. Life expectancy after diagnosis is only about three to five years. The only cure is a lung transplant.

"Although the results were significant, this was a small study and we believe that a larger trial is warranted to confirm these promising results and also assess the drug's impact on the disease itself," says Horton. "We have some hope that this therapy may be able to slow the progression of IPF, but that would have to be tested in a larger study."

Provided by Johns Hopkins University School of Medicine