

Tamoxifen for the Treatment of Retroperitoneal Fibrosis

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The full report is titled “Brief Communication: Tamoxifen Therapy for Nonmalignant Retroperitoneal Fibrosis.” It is in the 17 January 2006 issue of *Annals of Internal Medicine* (volume 144, pages 101-106). The authors are E.F.H. van Bommel, T.R. Hendriks, A.W.L.C. Huiskes, and A.G.M. Zeegers.

What is the problem and what is known about it so far?

Retroperitoneal fibrosis (RF) is a rare condition in which hard scar tissue develops behind the abdomen. It develops because of cancer, inflammation, or for no apparent reason. It first causes symptoms of dull low back or abdominal pain. As it progresses, the scar tissue may press on the kidneys and blood vessels and cause bloody urine, leg pain, and blood clots. Treatment of RF is surgical removal of the tissue. However, because the tissue is extremely hard, it may not easily separate from organs and vessels. As a result, complete surgical removal is often impossible. For this reason, there is great interest in finding drugs to help treat RF. Tamoxifen is a drug that blocks the effects of the hormone estrogen. Reports suggest that tamoxifen is useful for treating RF. This information, however, comes from individual patients treated and monitored differently by different doctors. To be more sure that tamoxifen is safe and effective in treating RF, it would be helpful to have information from a larger group of patients who were treated and monitored similarly.

Why did the researchers do this particular study?

To see whether tamoxifen is safe and effective in a large group of patients with RF.

Who was studied?

19 patients with RF who were seen over 6 years at a Dutch medical center. Most of the patients were older men.

How was the study done?

The researchers assessed the patients’ symptoms. They also took blood tests to look for signs of inflammation and measured the size of the scar tissue using computed tomography (CT) scans. They then prescribed tamoxifen for the patients and measured changes in symptoms, signs of inflammation, and size of scar tissue every few months while the patients were taking the drug.

What did the researchers find?

Tamoxifen helped 14 of the 19 patients. Symptoms improved within weeks, and within months signs of inflammation decreased and the scar tissue shrunk. Retroperitoneal fibrosis returned in 1 patient after the drug was withdrawn but improved when therapy was started again. Of the 5 patients who did not improve while taking tamoxifen, 3 did well on a combination of drugs that suppress the immune system. None of the patients experienced any serious side effects.

What were the limitations of the study?

The researchers did not compare tamoxifen with surgery or other drugs. Therefore, the findings do not imply that tamoxifen is better than other treatments. The researchers studied the patients for only 2 years. Many patients with RF will take the drug for longer periods. It is possible that tamoxifen may be less safe or effective when taken over this longer time. Also, the findings did not provide information about why tamoxifen may be effective for RF.

What are the implications of the study?

Tamoxifen can be considered an option for the treatment of patients with RF. It is not yet known how long the drug should be taken to be most effective or how safe and effective it is over long periods of time and compared with other treatments.

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