

Ruxolinitib better at reducing myelofibrosis symptoms, study shows

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In a major advance in treatment, a multicenter study found that ruxolinitib did a better job than off-label chemotherapy drugs reducing the terrible symptoms associated with myelofibrosis, including pain, enlarged spleen, anemia, fever, chills, fatigue, and weight loss. The results were presented today at the annual meeting of the American Society of Hematology and Oncology in San Diego.

Myelofibrosis is a bone marrow disorder that disrupts the body's normal production of blood cells, resulting in extensive scarring in the bone marrow. Patients tend to be over age 50.

"For years, researchers have struggled to find effective medications to help patients struggling with symptoms of this chronic pre-leukemia condition," says lead investigator Ruben Mesa, M.D., of Mayo Clinic in Arizona. There are several thousand new myelofibrosis patients in the U.S. every year.

Only about 10 percent of myelofibrosis patients are eligible for a <u>bone</u> marrow transplant and <u>chemotherapy</u> often falls short, Dr. Mesa says. A handful of off-label chemotherapy drugs have been modestly helpful, he says.

A randomized, double-blind clinical trial, known as the COMFORT-1 study, showed that ruxolinitib reduced spleen size by more than 35 percent in almost all of the 154 patients studied. An enlarged spleen, caused by sequestered over-proliferating blood cells, causes discomfort



and can also lead to the need for blood transfusions and further <u>medical</u> <u>complications</u> for patients.

In the study, patients treated with ruxolinitib also reported less severe symptoms than those reported by patients treated with a placebo.

A tandem randomized investigation in Europe, known as the COMFORT-2 study, showed that ruxolinitib also was more effective at reducing symptoms than the best available chemotherapies -- specifically, the off-label drugs doctors have been offering.

"The studies confirmed the drug is very effective. As a representative of a particular class of molecular inhibitors called JAK2 inhibitors, which are now being widely studied, ruxolinitib suggests this category will continue to be promising for myelofibrosis," Dr. Mesa says. Most surprising for researchers, however, was the ultimate comparison between the two clinical trials.

"When we looked at the control arms of the two studies we found that the best available chemotherapies we've been using up to this time are no more effective than a placebo," Dr. Mesa says. The comparison provided a wake-up call, he adds. "This was a unique piece of information we hadn't had in the past, and it offers further incentive and pressure to find better therapies for patients."

Provided by Mayo Clinic

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