Myelodysplastic syndrome treated with deferasirox shows beneficial iron reduction

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Researchers at Moffitt Cancer Center and colleagues at six other institutions have recently tested a treatment for patients with myelodysplastic syndrome, or MDS, a blood-related malignancy that involves the ineffective production of blood cells, leaving patients anemic and in need of frequent blood transfusions. Because the body has no natural means to reduce iron that accumulates from repeated transfusions, patients' organs can become iron overloaded, leading to heart failure, liver injury, susceptibility to infection, and other complications. Bone marrow failure and conversion to acute leukemia may occur in patients with MDS, necessitating bone marrow transplantation. The disease can be caused by chemotherapy and radiation treatment for cancer.

Deferasirox, the drug tested in the clinical trial, was aimed at reducing the elevated and risky iron levels that accompany the red blood cell transfusions. The study drug was received by 173 patients.

"The majority of patients with MDS require red blood cell transfusions for their anemia, but this leaves them at risk for iron overload," said study co-author Alan F. List, M.D., Moffitt's executive vice president and physician-in-chief. "Organ complications can arise for transfusion-dependent patients with MDS."

The two most serious complications for those with MDS, once called pre-leukemia, are infection and iron overload from transfused red blood cells .

When Robin Roberts, ABC's "Good Morning America" co-anchor, announced on the air in June that she has MDS and would undergo a bone marrow transplant, MDS was brought into sharp focus for Americans. At the time of her announcement, Roberts had been free of breast cancer for five years and had undergone chemotherapy and radiation.

The clinical trial results, published in a recent issue of the *Journal of Clinical Oncology*, show that deferasirox reduced iron (serum ferritin) by 23 percent in 53 percent of the patients who completed 12 months of treatment, reduced the blood iron in 36.7 percent of patients who had been on the treatment for two years, and patients who had been on the treatment for three years saw a 36.5 percent reduction in serum ferritin.

The drug also reduced labile plasma iron (LPI), called the most toxic aspect of nontransferrin-bound iron that accumulates after the body's iron storage capacity has been saturated.

"Overall, this study demonstrated improvements in iron parameters in a group of heavily transfused lower risk patients with MDS," explained List, whose research focuses on the biology of MDS and potential treatments. "A randomized trial is warranted to better ascertain the clinical impact of deferasirox therapy in lower risk patients with MDS."

Provided by H. Lee Moffitt Cancer Center & Research Institute