MedicalXpress

Novel drug prevents common viral disease in stem-cell transplant patients, study finds

September 25 2013, by Rob Levy

A new drug can often prevent a common, sometimes severe viral disease in patients receiving a transplant of donated blood-making stem cells, a clinical trial led by researchers at Dana-Farber Cancer Institute and Brigham and Women's Hospital indicates.

In a paper in the Sept. 26 issue of the *New England Journal of Medicine*, the researchers report that patients who took the drug CMX001 shortly after transplant were far less likely to develop cytomegalovirus (CMV) infection than were patients who took a placebo. CMV disease is a common source of illness in transplant patients and can cause pneumonia, diarrhea and digestive tract ulcers, or other problems. Although some antiviral drugs, when administered at the earliest signs of CMV infection, often forestall CMV disease, they often cause kidney dysfunction or impair patients' ability to make new blood cells.

"With current agents, between 3 and 5 percent of allogeneic [donor] transplant patients develop CMV disease within six months of transplantation, and a small number of them may die of it," says study lead author Francisco Marty, MD, of Dana-Farber and Brigham and Women's. "There clearly is a need for better treatments with fewer adverse effects. This clinical trial examined whether the disease can be prevented, rather than waiting for blood tests to show that treatment is needed."

CMV is a herpes-like virus that infects the majority of Americans by adulthood. Most often, it is held in check by the immune system and

produces no symptoms. Most people are unaware they are infected, Marty explains.

In stem-cell transplant patients, however, the immune system – based in the blood-making tissue of the bone marrow – is replaced with donor blood-making cells after patients receive high doses of chemotherapy. During this transition period, long-dormant viruses like CMV may have an opportunity to become active. The result can be CMV disease.

The new, phase II clinical trial involved 230 hematopoietic (blood-making) stem cell transplant recipients at 27 treatment centers across the United States. They participants were randomly assigned to receive either oral CMX001 (at varying doses) or a placebo, which they began taking after the transplanted cells engrafted, or took root in the bone marrow – which usually occurs two to three weeks after the transplant procedure. They took the pills for 9-11 weeks.

Only 10 percent of the patients who took 100 mg of CMX001 twice a week had a "CMV event" – defined as CMV disease that affects the lung, digestive tract, or other organs, or a detectable amount of CMV in the blood at the end of treatment – the investigators found. By contrast, CMV events occurred in 37 percent of those who received a placebo. The most common adverse side effect to CMX001 was diarrhea.

"The results show the effectiveness of CMX001 in preventing CMV infections in this group of patients," Marty says. "Because CMX001 is known to be active against other herpes viruses and against adenoviruses that sometimes affect transplant patients, it may be useful as a preventive or treatment agent for those infections as well."

Provided by Dana-Farber Cancer Institute