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The full report is titled “Reversibility of Cirrhosis in Patients Cured of Thalassemia by Bone Marrow Transplantation.” It is in the 7 May 2002 issue of *Annals of Internal Medicine* (volume 136, pages 667-672). The authors are P Muretto, E Angelucci, and G Lucarelli.

Reversibility of Liver Scarring in Patients Who Are Cured of Thalassemia

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

Thalassemia is a hereditary disease in which the bone marrow produces red blood cells that have a shorter life span than normal red blood cells. Patients with this disease accumulate iron in their bodies because they require frequent blood transfusions throughout their lives. The transfused red blood cells eventually break apart and release their contents (including iron) into the bloodstream. The liver removes excess iron from the bloodstream and stores it in liver tissue. Excessive accumulation of iron damages the liver and causes scar tissue to form. Accumulation of scar tissue causes a condition known as cirrhosis. Other diseases also cause cirrhosis, particularly hepatitis C virus (HCV) infection and severe alcoholism. For many years, doctors have believed that cirrhosis is permanent, perhaps because the conditions that cause it are usually permanent. Recently, doctors have been able to cure thalassemia by transplanting bone marrow from a normal donor. Since cured patients no longer need transfusions, much less iron enters their bodies, and iron may leave the liver and reduce the stimulus to form scar tissue. Many patients with thalassemia already have cirrhosis before they undergo bone marrow transplantation; therefore, it is possible to see whether cirrhosis decreases after successful treatment of thalassemia.

Why did the researchers do this particular study?

To see what happens to the liver when patients with thalassemia complicated by cirrhosis undergo bone marrow transplantation.

Who was studied?

6 patients (selected from 491 patients with thalassemia cured by bone marrow transplantation) whose liver disease improved after treatment. All patients had excessive stores of iron in the liver and were infected with HCV.

How was the study done?

Patients who had undergone bone marrow transplantation received drugs and had blood drawn to remove as much iron as possible from the body. They also received treatment for HCV infection. The doctors performed biopsies of the liver before and after successful bone marrow transplantation. Experts in liver disease examined the biopsy specimens under a microscope and measured the amount of scar tissue (or cirrhosis) without knowing who had the biopsy or when it was done.

What did the researchers find?

In every case, the amount of iron in the liver decreased substantially after thalassemia was cured. In addition, the amount of scar tissue found in the liver decreased in all patients, and cirrhosis improved.

What were the limitations of the study?

The number of patients studied was very small, and participants were selected from a larger group of patients who were cured of thalassemia specifically because their liver disease had improved with treatment. As a result, it is impossible to tell if this therapy would work for all patients with thalassemia.

What are the implications of the study?

The most important implication is that formation of scar tissue in the liver is not necessarily permanent. Reduction of scar tissue after successful treatment may occur in other diseases and in other organs.

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