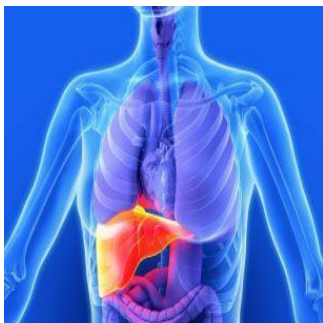


## Liver Transplantation for Patients with Genetic Liver Conditions Has High Survival Rate



Patients faced with the diagnosis of a life-threatening liver disease have to consider the seriousness of having a liver transplant, which can be a definitive cure for many acquired and genetic liver diseases. Among the main considerations are the anxiety of waiting for a donor organ, the risks associated with the transplant operation, and the chance that the transplant procedure will not achieve the desired result. There is also the six-figure cost of the procedure and accompanying patient care, all of which may not be completely covered by health insurance. But, according to a study appearing in the April issue of the *Journal of the American College of Surgeons*, researchers at the David Geffen School of Medicine, University of California–Los Angeles (UCLA) found that liver transplants are worth the risk for people who have genetic liver conditions.

The study is a first-of-its-kind, single-institution comparison of outcomes for both pediatric and adult patients undergoing liver transplantation for lethal genetic syndromes. Researchers found that children with genetic disorders that cause fibrosis, cirrhosis, and other liver conditions, which can affect other organs, have a good chance of still being alive five years, even 20 years after a liver transplant operation. Adults with these types of conditions also have high survival rates.

"If we had not transplanted these patients, long term, they would have died," said study lead author, Henrik Petrowsky, MD, who was an attending transplant surgeon and assistant professor of surgery at UCLA during the time the study was conducted.

Patients with genetic liver conditions are different from those who developed liver diseases from a hepatitis C infection or alcoholism. First, this patient population is less common. Dr. Petrowsky estimates that only about 9 percent of liver transplants in children and only 2 percent in adults are performed as a treatment to correct genetic conditions. Second, the severity of genetic liver disease may not be as obvious. Since donated livers are allocated according to who needs them most, people with genetic liver diseases often have to be given extra consideration when weighing how long they can wait for a donor organ.

Although the genetic defect permeates every cell in the affected patient's body, it expresses mainly in the liver. However, certain disorders could also affect other organs. During a transplant, when the old liver is replaced with a new one "it's almost a form of gene therapy," explains Dr. Petrowsky, who is now vice chair of the department of visceral and transplant surgery at the University Hospital Zurich in Switzerland. "The genetic defect is still in every cell, but since it's mainly expressed in the liver, the genetic disorder is corrected by the transplanted liver which does not harbor the genetic defect."

Dr. Petrowsky's team wanted to evaluate the impact of the transplant procedure on patients' outcomes, especially because patients families go through so much before a transplantable liver can be procured.

Therefore, the investigators looked at patient records from the UCLA liver transplant database for 74 children and 78 adults who had received liver transplants between 1984 and 2012 to correct genetic disorders. For 68 percent of those patients, the genetic disorder led to cirrhosis, or scarring of the liver, making liver transplantation their only hope for survival.

Five years later, 89 percent of children in the study were still alive, and 77 percent were still alive 20 years later. For adults, 73 percent were still alive after five years and 50 percent were still alive after 20 years.

"Without a transplant, the five-year survival rates are below 5 percent, depending on the severity of the liver disease," Dr. Petrowsky said.

Only one pediatric patient and one adult patient had a recurrence of the underlying genetic disease. The pediatric patient died 12 years after the transplant. The adult had another liver transplant and was still alive 22 years later. "For certain genetic conditions, replacing the liver is not enough," Dr. Petrowsky explained, because "other tissues and organs are expressing the mutated protein. So the discussion now is should we do a combined liver and bone marrow transplantation in these rare cases to improve the outcome?"

Only 13 percent of adult patients and 22 percent of pediatric patients required another transplant. For pediatric patients, the primary reasons for the additional procedure were blood clots in the hepatic artery or the body rejected the new organ. The majority of adults who needed another transplant had primary non-function, meaning the transplanted organ simply didn't work.

Despite the complications, Dr. Petrowsky said the high survival rates also imply that liver transplantation is currently the best available treatment for patients with genetic liver disease. "Liver transplantation for lethal genetic syndromes represents a model of personalized genomic medicine by providing gene therapy through solid organ transplantation," the authors wrote. Additionally, genetic testing could be used to not only confirm the cause of liver disease, but also predict it in people who have certain genetic markers. The results of that finding could be used to guide treatment decisions.

The high survival rates also make up for the risks and the costs of liver transplantation, which could be as high as \$577,000 according to the United Network of Organ Sharing.\* "Transplantation saves lives," Petrowsky said. "Many of the patients go on to live a good life. We have pediatric patients who were transplanted as infants. If they had not received a transplant, they would not have survived. Many of them are alive more than 20 years after transplant, are married, and some of them have even kids, and have great careers," he concluded.

Other participants in the study included F. Charles Brunicki, MD, FACS; Voon Meng Leow, MD; Robert S. Venick, MD; Vatche Agopian, MD; Fady M. Kaldas, MD; Ali Zarrinpar, MD, PhD; Daniela Markovic, MS; Sue V. McDiarmid, MD; Johnny C. Hong, MD, FACS; Douglas G. Farmer, MD, FACS; Jonathan R. Hiatt, MD, FACS, and Ronald W. Busuttil, MD, FACS.

\* *Financing a Transplant. United Network for Organ Sharing. Available at <http://www.transplantliving.org/before-the-transplant/financing-a-transplant/the-costs/>. Accessed April 4, 2013.*

Source: [American College of Surgeons](#)

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