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Abstract:

BACKGROUND: Crigler-Najjar syndrome type 1 (CNS1) is characterized by severe unconjugated hyperbilirubinemia from birth, caused by total failure of UDP-glucuronyltransferase activity. Only orthotopic liver transplantation (OLT) offers the prospect of cure. However, because the onset of neurologic deficits is unpredictable, timing of OLT remains difficult.

METHODS: In our transplant center, 3 patients underwent early OLT for CNS1. Two of them (7 yr, 12 yr) showed mild to moderate neurologic deficits only few weeks before OLT, another patient (4 yr) had no signs of bilirubin encephalopathy. All patients required extensive phototherapy to control bilirubin levels. Thus, OLT was performed shortly after the onset of neurologic symptoms or as a prophylactic procedure, respectively.

RESULTS: OLT was uneventful in all recipients. One of the symptomatic patients (7 yr) completely recovered from neurologic deficits at 36 months on OLT, whereas the other patient (12 yr) significantly improved symptoms at 27 months of OLT. These patients, including the 4-year-old boy, attend school at appropriate grades now.

CONCLUSIONS: Irreversible brain damage (kernicterus) may occur in the course of CNS1 disease. Because no alternative treatment options are available at this time, OLT should be performed as a preventive procedure to counteract severe CNS-related complications.