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Predictive factors of response to non-transplant treatment strategies in progressive familial intrahepatic cholestasis type II

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Abstract

Purpose: Progressive familial intrahepatic cholestasis type II (PFIC-II) is a defect of bile salt exporter protein (BSEP) at the canalicular surface of the hepatocytes. The defect of BSEP leads to progressive liver injury and eventually cirrhosis. Thetreatment for PFIC-II includes liver transplantation (LT), UrsoDeoxyCholic Acid (UDCA) and biliary diversion (BD). There are no predictive factors to assess the responsiveness of patients to non-transplant modalities. Methods: Retrospective analysis of 33 PFIC-II patients was done. Diagnostic criteria were compatible clinical presentation with either confirmatory genetic analysis or the absence of BSEP on immuno-histochemistry. The need for LT was taken as a poor outcome while maintenance on non-LT treatment was as good. The UDCA and BD response was assessed on the following parameters. Normalisation Remission of clinical manifestations, normal liver enzymes and serum bile acids. Time to normalization (TTN) - Duration from start of UDCA or BD until biochemical normalization Duration of normalization (DOR) - Duration of time for which normalization was sustained Results: 33 PFIC-II children included, LT (n=20) and non-LT (n=13) groups comparable in terms of age and sex. The two groups differed significanty for the age at first presentation, history of neonatal jaundice & ALT levels. 4 of 5 (80%) with homozygous mutations needed a LT. BSEP staining positive was seen only in 6 and canalicular in 3. 1/6 with cytoplasmic staining needed LT. 7/33 responded to UDCA, 3 of these transiently and 4 with ongoing response. The mean TTN was 16.7 +/- 3.3 months and there was no significant difference between the transient and sustained responders. The mean DOR in the sustained responders is 41.2+/-9.4 months while in the transient responders it was 70+/-7.5 months. Conclusions: 30% of PFIC - II patients achieve normalization with non-LT treatment, while disease progress may not be affected. Prognostic factors identified for good response to non-LT management in PFIC-II are age at presentation >1 year, no neonatal

jaundice, high ALT, absence of homozygous mutation and presence of BSEP on immunohistochemistry. The trial with UDCA to assess response should be minimum 2 years. The response that is seen may not be permanent. (Table Presented).

Drug terms

protein ursodeoxycholic acid liver enzyme nitrogen 13 bile salt

Disease terms

intrahepatic cholestasis **liver disease** newborn jaundice liver cirrhosis liver injury

Other terms

liver **transplantation** human patient mutation immunohistochemistry staining liver transplantation liver cell parameters genetic analysis diagnosis remission bile acid blood level child

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