Clinical outcomes and validation of UDCA response scores in Korean with primary biliary cholangitis

사랑의 병원 내과[1]

박혜연[1]

Background/Aims: Ursodeoxycholic acid (UDCA) Response Score (URS) was developed to identify poor responders to UDCA treatment before treatment, in order to offer timely and proactive intervention. However, external validation of the URS in the Asian population is warranted.

Methods: A total of 173 Asian patients diagnosed with primary biliary cholangitis (PBC) between 2007 and 2016 at 7 academic institutions in South Korea who started UDCA treatment were analyzed to validate performance of URS. UDCA response was defined as an alkaline phosphatase (ALP) level less than 1.67 times the upper limit of normal (ULN) after one year of UDCA treatment. In addition, prognostic performance of URS for liver-related events, defined as newly developed hepatic decompensation (variceal bleeding, ascites, or hepatic encephalopathy) or hepatocellular carcinoma was evaluated.

Results: After one year of UDCA treatment, 133 patients (76.9%) achieved UDCA response. UDCA response rate was 98.7% of those with URS ≥1.41 (n=76) and was 78.8% for those with URS <1.41 (n=97). The area under the receiver operating characteristic curve (AUROC) of URS for prediction of UDCA response was 0.84 (95% confidence interval (CI): 0.78 – 0.88). During a median follow-up of 6.5 years (range 1.0 – 14.1 years), liver-related events developed in 18 patients (10.4%). Among 117 patients with PBC stage I-III by histological evaluations, the liver-related events-free survival rate differed by URS; 100% at 5-years for URS ≥1.41 and 86.5% at 5-years for URS <1.41 (p=0.005).

Conclusions: URS demonstrated good performance in predicting the UDCA treatment response in Asian PBC patients. In addition, the risk of liver-related events differed by URS in PBC stage I-III population. URS can be a tool to predict response and clinical outcome in Asian patients with PBC.

PBC Figures

Figure 1 Study population

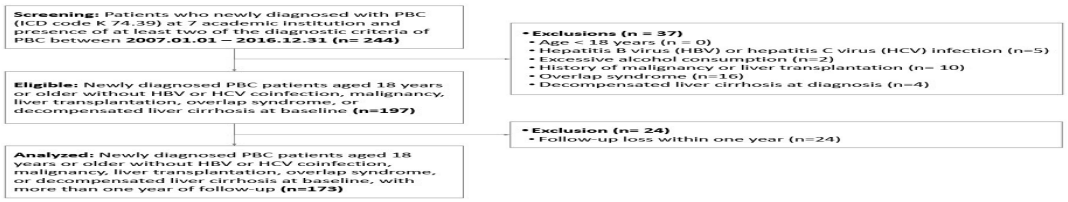


Figure 2 Probability of liver-related event free survival of the patients with available histologic data stratified by fibrosis stage (n=125)

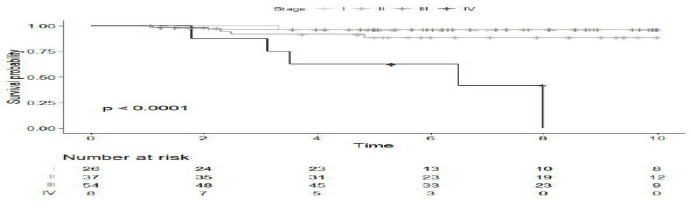
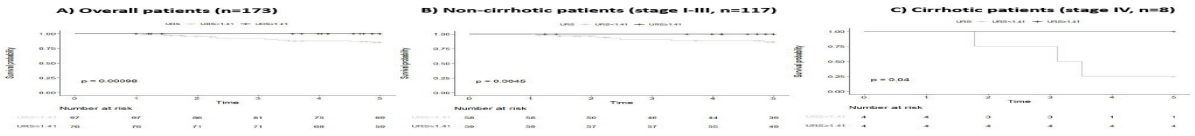


Figure 3 Probability of liver-related events free survival stratified by URS score



URS 1.41 : cut-off value of predicting ALP endpoints (=1.67 X ULN)

Supplementary Figure 1 AUROC for prediction of response to UDCA calculated with URS with ALP endpoint <1.67 X ULN