Novel prognostic model for primary sclerosing cholangitis: the importance of including biochemical values

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Introduction:
Primary sclerosing cholangitis (PSC) is a chronic, cholestatic liver disease, without effective drug treatment options. The only curative option is liver transplantation (LT). It is important to be able to make an indication of prognosis, for purposes of patient counseling, management and adequate timing of LT. Aim of this study was to create a prognostic model consisting of disease phenotypical as well as biochemical variables.

Methods:
692 PSC patients were identified in a large population based PSC cohort from the Netherlands. Variables of PSC phenotype, biochemistry results and long term follow-up data were retrieved from patient records. Clinical endpoints were development of cholangiocarcinoma, LT, or PSC-related death. Laboratory values were transformed by log transformation, missing values were imputed by multiple imputation. To calculate the prognostic index (PI), Cox proportional hazards model was developed and internally validated with bootstrap.

Results:
The median follow-up time was 85 months (range 0-468 months). All phenotypical variables and biochemistry results were considered for the model. After variable selection by LASSO, multivariable Cox models were fitted, and parameters estimated from 20 imputation datasets were averaged. Model performance was assessed with C-statistics and adjusted for optimism with 1000 times bootstrap.

Conclusion:
By using a population based PSC cohort, we were able to create a prognostic model based on disease phenotypical and biochemical variables. Internal validation using bootstrap showed adequate performance. The inclusion of biochemistry could facilitate the dynamic prediction of PI over time.