

Long-Term Consequences

PERSISTENT ELEVATION OF sBA LEVELS INCREASES THE RISK FOR LIVER TRANSPLANT IN PATIENTS WITH PFIC



Preservation of Native Liver

In an analysis of data from the NAPPED consortium, patients who achieved serum bile acid (sBA) concentrations below certain thresholds following surgical biliary diversion (SBD) had longer native liver survival.^{1,2}

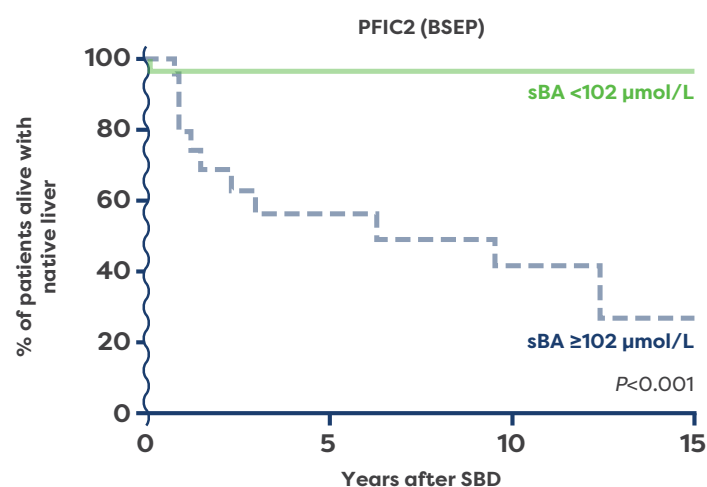
PFIC2 (BSEP)

sBA <102 $\mu\text{mol/L}$ was associated with native liver survival of 15 years following SBD¹

PFIC1 (FIC1)

sBA <65 $\mu\text{mol/L}$ was associated with higher native liver survival at 15 years following SBD²

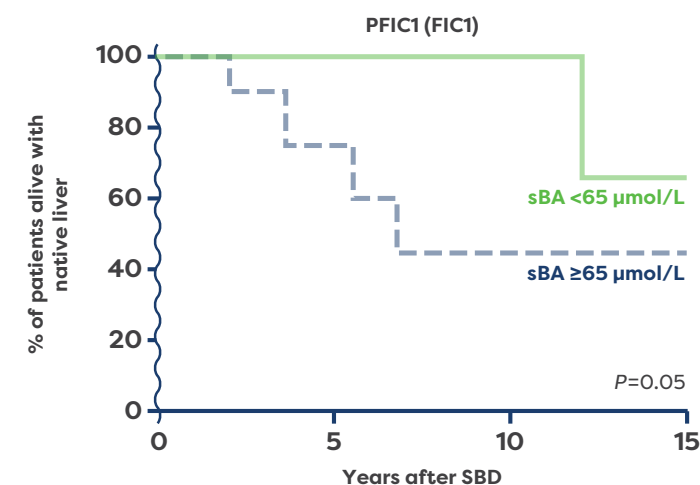
Native liver survival after surgical biliary diversion, stratified by post-surgical sBA levels^{1,2}



Number at risk

<102 $\mu\text{mol/L}$	27	23	16	9
$\geq$ 102 $\mu\text{mol/L}$	20	8	5	1

Based on a multicenter, retrospective cohort study that included 264 patients aged 0 to 18 years with homozygous or compound heterozygous pathological *ABCB11* mutations. Median age at time of SBD was 2.3 (1.2–4.7) years (n=61). Median follow-up after SBD was 8.4 (1.6–12.0) years.¹



Number at risk

<65 $\mu\text{mol/L}$	12	7	5	2
$\geq$ 65 $\mu\text{mol/L}$	11	5	1	1

Based on a multicenter, combined retrospective and prospective study that included 130 patients aged 0 to 18 years with compound heterozygous or homozygous predicted pathogenic *ATP8B1* variants. Median age at time of SBD was 5.9 years (n=62). Median follow-up after SBD was 5.3 years.²

Data in pediatric patients show that lowering sBA through surgical intervention can impact transplant-free survival.

Given the implications of bile acid and long-term outcomes, what could this mean for your adult patients with PFIC?

BSEP=bile salt export pump; FIC1=familial intrahepatic cholestasis 1; NAPPED=Natural course and Prognosis of PFIC and Effect of biliary Diversion.

References: 1. van Wessel DBE, Thompson RJ, Gonzales E, et al; NAPPED Consortium. Genotype correlates with the natural history of severe bile salt export pump deficiency. *J Hepatol.* 2020;73(1):84-93. doi:10.1016/j.jhep.2020.02.007 2. van Wessel DBE, Thompson RJ, Gonzales E, et al; NAPPED Consortium. Impact of genotype, serum bile acids, and surgical biliary diversion on native liver survival in FIC1 deficiency. *J Hepatol.* 2021;74(2):892-906. doi:10.1002/hep.31787