Pearls for Assessing Treatment Response to First-line Therapy in PBC



Assessing UDCA Treatment Failure Is Critical in PBC as up to 40% of Patients Have Suboptimal Biochemical Responses

Determining treatment failure is essential to avoid potential therapeutic delays with new second-line treatment options (e.g., PPAR agonists).

Definition for treatment failure/inadequate response to UDCA varies by assessment

- POISE criteria have been most commonly used in clinical trials

Assessment	Time (mo)	Definition of Treatment Failure
Rochester	6	ALP ≥2 × ULN or Mayo score ≥4.5
Paris-I	12	ALP ≥3 × ULN or AST ≥2 × ULN or bilirubin >1 mg/dL
Paris-II	12	ALP ≥1.5 × ULN or AST ≥1.5 × ULN or bilirubin >1 mg/dL
POISE	12	ALP >1.67 × ULN or bilirubin >1 × ULN
Toronto	24	ALP >1.67 × ULN
Rotterdam	12	Bilirubin ≥1 × ULN and/or albumin <1 × ULN

Consider incorporating continuous scoring systems

- Improve prediction of death and liver transplantation risk

Assessment	Time (mo)	Scoring Parameters
GLOBE	12	■ Bilirubin, ALP, albumin, platelet count at 12 months ■ Age at baseline
UK-PBC	12	■ Bilirubin, ALP, and AST (or ALT) at 12 months ■ Albumin and platelet count at baseline

Consider utilizing the UDCA Response Score (URS)

- Validated tool to predict UDCA response

Variables Associated with a Lower Probability of UDCA Response		
↑ ALP	↓ Amino-transferases	↑ Treatment delay from diagnosis to start of UDCA treatment
↑ Total bilirubin	Younger age	Worsening ALP from the time of diagnosis

ABBREVIATIONS

ALP: alkaline phosphatase
ALT: alanine aminotransferase
AST: aspartate aminotransferase
PBC: primary biliary cholangitis
mo: month
UDCA: ursodeoxycholic acid
ULN: upper limit of normal

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