EFFICACY AND SAFETY ANALYSIS OF OBETICHOLIC ACID IN PRIMARY BILIARY CHOLANGITIS: REAL-LIFE DATA

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4CPS-216

A05- BILE AND LIVER THERAPY

BACKGROUND AND IMPORTANCE

Obeticholic acid (OCA) is an **orphan drug** for patients with **primary biliary cholangitis** (**PBC**), a rare autoimmune disease, who do not respond adequately to treatment with **ursodeoxycholic acid** (UDCA) or do not tolerate it.

AIM AND OBJECTIVES

To **evaluate the efficacy and safety of OCA** in patients with **PBC.**

MATERIAL AND METHODS

Descriptive and retrospective study.
Patients who received OCA from **January-2021 to April-2023**

Data collected
Sex
Age
Previous treatment with UDCA
Alkaline phosphatase (ALP)
Gamma-glutamyl transferase (GGT)
Total bilirubin (Bt)
Aspartate aminotransferase (AST)
Alanine aminotransferase (ALT)
Adverse effects

At the **start** of treatment with OCA, at **6 months** and at **12 months**

According to the pivotal drug trial, **treatment response** was defined as:

- **ALP <1.67 x ULN**,
- **Bt value within the normal range** AND
- a **decrease from baseline ALP value of at least 15%**

RESULTS

N=30
87% women
Median age: 66 years
97% were on treatment with UDCA

Median values and percentile 25-75 are shown

	Baseline	6 months	12 months
ALP	333,5 (242-453,5)	295,5 (187-428)	252,5 (162-332,2)
Bt	0,6 (0,5-0,7)	0,7 (0,5-0,8)	0,6 (0,4-0,77)
GGT	136 (84,5-279,5)	82,5 (39,5-187,5)	56 (22,2-113,2)
AST	36,5 (33,5-45,7)	32,5 (29-49,5)	35 (28-45)
ALT	40,5 (28,2-61,5)	30,5 (23-46)	29,5 (23-43,7)

A **reduction of ALP>15%** was achieved in 15 (**50%**) and 16 patients (**53%**) at 6 and 12 months, respectively.
29 patients (**97%**) had **bilirubin in the normal range** at 6 months, and all (**100%**) at 12 months.
ALP<1.67xULN was obtained in 7 (**23%**) and 11 (**37%**) patients at 6 and 12 months, respectively.

Overall, **4 patients (13%) fulfilled the 3 pivotal trial conditions at 6 months** and **8 patients (26%) at 12 months**. Adverse reactions reported were: pruritus in 14 patients (47%) and fatigue in 1 (3%)

CONCLUSION AND RELEVANCE

Based on clinical trial endpoints, **OCA achieved modest results at 6 months**, which **doubled one year after initiation of treatment**. Further studies are needed to assess long-term benefit.