Real World Experience with Obeticholic Acid for Primary Biliary Cholangitis: A Case Series

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Real World Experience With Obeticholic Acid for Primary Biliary Cholangitis: A Case Series

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INTRODUCTION

Obeticholic acid (OCA) is a farnesoid X receptor agonist that serves as a novel agent for the treatment of primary biliary cholangitis (PBC). Its efficacy was demonstrated during the POISE trial where 46% of patients achieved the composite endpoint and 77% of patients achieved a reduction of alkaline phosphatase (AP) by ≥15%. We present a single-center real-world experience with OCA for PBC.

CASES

Since August 2016 our center has treated ten PBC patients with OCA. Patient characteristics are in Table 1. The mean patient age was 58.7. The median duration of therapy with OCA was 14.5 months. One patient did not use concurrent therapy with ursodiol due to cost.

The trends in AP are shown in Table 2 and Figure 1. Outcomes are delineated in Table 3.

Two patients (20%) met the composite endpoint as delineated in the POISE trial. Seven patients (70%) had an improvement in AP by at least 15%. Only three of the patients had a dose titration from 5 mg to 10 mg. Eight patients had at least a mild transient rise in AP after initial improvement, one of which had an AP increase above their pre-treatment baseline.

One patient had clinically significant acute hyperlipidemia with a serum LDL level of 367 mg/dL. No other adverse events were noted including worsened pruritus.

Table 1. Patient Characteristics

Patient	Age	Gender	Diagnosis Time (Years Prior to OCA)	Diagnosis Method	Cirrhosis (CTP Score)?	Duration OCA Therapy (Months)	Concurrent Ursodiol?	Adverse Events	Other Notes
1	54	F	14	Biopsy, +AMA	Yes (A)	17	Yes	None	None
2	67	F	11	+AMA	No	16	Yes	None	None
3	52	M	0	Biopsy, +AMA	No	25	No	Severe Hyperlipidemia	History of heart failure and aortic valve replacement during therapy
4	66	F	4	Biopsy, -AMA	Yes (A)	20	Yes	None	History of CKD4, Missed one month therapy due to medication delivery issue
5	67	F	1	Biopsy, +AMA	No	24	Yes	None	None
6	34	F	1	Biopsy, +AMA	Yes (A)	13	Yes	None	None
7	54	F	1	+AMA	No	12	Yes	None	None
8	79	F	15	Biopsy, -AMA	Yes (A)	8	Yes	None	None
9	63	F	20	Biopsy, +AMA	No	9	Yes	None	Overlap Autoimmune Hepatitis, Previously on OCA 2 months stopped for pruritis
10	51	F	10	Biopsy, +AMA	No	11	Yes	None	None

Table 2. Alkaline Phosphatase Trends During Treatment with Obeticholic Acid

	Time (Months)	Patient 1	Patient 2	Patient 3	Patient 4	Patient 5	Patient 6	Patient 7	Patient 8	Patient 9	Patient 10
Pre-Treatment	0	209	202	875	277	272	252	350	330	259	332
	1		224			215	200				
	2		219	564	218	175	185	311	317	206	180
	3	172							300		
	4		213		299		188	300	254		
	5			657							197
Dose 10 mg	6	150		659	251					218	
	7			622			171	285			
	8		205	588							
	9				277						
	10	156				158		236			
	11		211				158	249			
	12	156		598							
	13					213					
	14										
	15			1311	257	93					
	16										
	17					155					
	18										
	19										
	20										
	21										
	22			1266							

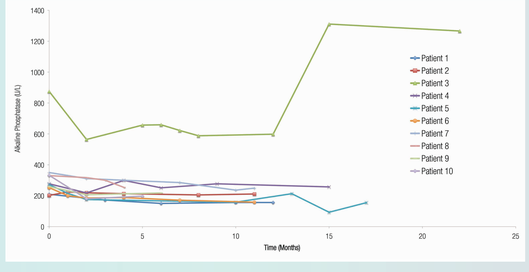
Table 3. Individual and Composite Endpoints with OCA Therapy*

**Endpoints defined by POISE clinical trial for patient outcomes at the end of 12 months therapy.*

Patient	AP <1.75 x ULN	AP Decrease 15% From Baseline	Normal Total Bilirubin	Composite Outcome
1	Yes	Yes	Yes	Yes
2	No	No	Yes	No
3	No	No	No	No
4	No	No	Yes	No
5	Yes	Yes	Yes	Yes
6	Yes	Yes	No	No
7	No	Yes	Yes	No
8	No	Yes	Yes	No
9	No	Yes	Yes	No
10	Yes	Yes	No	No

Figure 1. Alkaline Phosphatase Trends During Treatment with Obeticholic Acid*

** T = 0 represents AP level at treatment initiation.*



DISCUSSION

Our real-world experience with OCA for PBC continues to shed new information on its treatment efficacy and safety. In light of recent concerns regarding the safety of OCA in patients with cirrhosis, our findings demonstrate that it is well tolerated in this population.

Despite the fact that our much of cohort did not meet the primary endpoint established in the POISE trial, overall there was a positive effect on the AP for a significant portion of our patients. Our data is limited by the small sample size and duration of therapy. Uniquely, we were able to show the successful use of OCA in a patient with chronic kidney disease.

The two patients who met the composite clinical endpoints did so at 2 and 6 months of therapy without titration of the dose to 10 mg suggesting effects are likely to be seen early. Interestingly, both of these patients had also had compensated cirrhosis.

One patient had a significant rise in AP during his therapy. However, this patient was not taking concomitant ursodiol and has significant medical comorbidities such as congestive heart failure that could contribute to this rise. He was eventually lost to follow up with questionable compliance.

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