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Real-world outcomes associated with lomitapide use in patients with homozygous familial hypercholesterolemia: A retrospective database analysis

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Statement of the Problem: Homozygous familial hypercholesterolemia (HoFH) is ultra-rare and hard to treat, with high LDL-C and premature major adverse cardiovascular events (MACE). No randomized trials, few real-world studies, and no published lomitapide (LOM) studies address MACE in HoFH, despite LOM being indicated as an adjunct therapy to diet and other lipid-lowering treatments for HoFH. This study presents for the first time MACE rates in LOM-treated patients. Methodology & Theoretical Orientation: Optum claims data (2012-2016, ~25 million covered lives) were combined with LDL-C from Optum and a pharmaceutical patient support program. LDL-C, and rates of MACE and hospitalization were assessed in HoFH patients for 1 year before drug initiation, during LOM treatment, and 1 year after LOM discontinuation (in those who discontinued). Patients were stratified based on LDL-C response to LOM. Findings: 62 patients started LOM and had baseline and follow-up LDL-C. LOM use was associated with LDL-C reductions and a trend to lower hospitalizations and MACE rates. Nearly 2/3 of patients (40 of 62) had a sustained response to LOM (>10% LDL-C reduction for >60 days), of whom 27 later discontinued LOM, with the expected subsequent worsening of outcomes. Conclusion & Significance Consistent with clinical trials, LOM significantly reduces LDL-C in real-world HoFH patients. Although data are limited by the rarity of HoFH, there was clinically meaningful (albeit not statistically significant) decrease in MACE and hospitalization with LOM treatment, and, as expected, there was a trend toward loss of LDL-C and MACE/hospitalization benefits after LOM discontinuation.

	All Patients (N=62)			Sustained Responder* (N=40) (Among All Patients)			Discontinuers (N=27) (Following Sustained Response)		
	Δ Diff (After - Before LOM initiation)	95% CI	% change	Δ Diff (After - Before LOM initiation)	95% CI	% change	Δ Diff (After - Before LOM discontinuation)	95% CI	% change
LDL-C, mg/dL	-54	[-68.8, -39.2]	-27%	-78.4	[−92.8, −63.9]	-38%	24.1	[−7.3, 55.5]	20%
Hospitalization rate, %	-0.13	[−0.31, 0.06]	-63%	-0.20	[-0.45, 0.06]	-75%	0.19	[−0.10, 0.48]	224%
MACE rate, %	-0.11	[−0.50, 0.28]	-19%	-0.22	[-0.65, 0.21]	-37%	0.38	[−0.15, 0.90]	452%

*Sustained responder are required to have an average post-index LDL-C level at least 10% lower than the baseline value. Additionally, individual post-index LDL reading should not be higher than the baseline level (with some initial fluctuation very close to the index date allowed, per visual examination of the trend). Lastly, the duration of LDL response period should last longer than 60 days.

Figure 1: LDL-C, Annual Hospitalization Rates, and MACE Rates in Patients with HoFH

Biography

Eliot A. Brinton is the President of the Utah Lipid Center in Salt Lake City. He is a founding board member of the National Lipid Association and of the American Board of Clinical Lipidology, of which he is also Past-President. He is a fellow of the American Heart Association and of the National Lipid Association, and past chair of the Clinical Lipidology Committee of the AHA. Dr. Brinton is widely published in the field of lipoprotein metabolism, having published original research articles in the New England Journal of Medicine, JAMA, and Circulation. He is Associate Editor of the Journal of Clinical Lipidology and Assistant Editor of the Journal of Obesity, and has served on the editorial boards of the Journal of Clinical Endocrinology and Metabolism and the Journal of Managed Care Pharmacy. Dr. Brinton has served on the faculty of the Rockefeller University, Wake Forest University and the University of Utah. He has won several awards and has held numerous leadership and advisory positions in scientific and governmental organizations and in the pharmaceutical industry.

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