ELIGIBILITY FOR PCSK9 TREATMENT IN 734 HYPERCHOLESTEROLEMIC PATIENTS REFERRED TO A REGIONAL ChOLESTEROL TREATMENT CENTER WITH LDL CHOLESTEROL ≥70 MG/DL DESPITE MAXIMAL TOLERATED CHOLESTEROL LOWERING THERAPY

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Background LDL cholesterol (LDLC) lowering has been revolutionized by PCSK9 inhibitors, Alirocumab (Praluent) and Evolocumab (Repatha), which have approved indications as an adjunct to diet-maximally tolerated cholesterol lowering therapy in heterozygous (HeFH) or homozygous (HoFH) familial hypercholesterolemia, and/or clinical atherosclerotic cardiovascular disease (CVD) where LDLC lowering is insufficient despite maximal tolerated therapy.

Methods We applied FDA approved and commercial insurance eligibility criteria for PCSK9 inhibitor use in 734 patients serially referred over 3 years who then received ≥2 months maximally tolerated LDLC lowering diet-drug therapy with follow up LDLC ≥70 mg/dl, as well as in 37 patients approved by commercial insurance for PCSK9 inhibitors. We obtained estimates of the percentage of patients with HeFH and/or CVD who meet FDA and commercial insurance eligibility for PCSK9 inhibitors using LDLC goal-based guidelines.

Results Of the 734 patients with LDLC ≥70 mg/dl after ≥2 months maximally tolerated LDLC lowering therapy, 220 (30%) had HeFH and/or CVD events with LDLC >100 mg/dl, meeting both FDA and commercial insurance criteria for PCSK9 inhibitor therapy. Sixty-six (9%) patients were statin intolerant, without HeFH or CVD events. Of the 37 patients whose PCSK9 inhibitor therapy was approved for coverage by medical insurance carriers, 34 (92%) had LDLC>100 mg/dl after ≥2 months on maximally tolerated LDLC lowering therapy. Sixteen (43%) of these 37 patients had HeFH without CVD (LDLC on maximally tolerated conventional treatment 181±48 mg/dl), 11 (30%) had CVD without HeFH (LDLC on maximally tolerated conventional treatment 122±22 mg/dl), and 8 (22%) had both HeFH and CVD (LDLC on maximally tolerated conventional treatment 204±56 mg/dl).
Conclusion Of the 734 patients referred for high LDLC treatment, with LDLC ≥70 mg/dl after ≥2 months on maximally tolerated therapy, 220 (30%) had HeFH and/or CVD with LDLC >100 mg/dl, meeting both FDA and insurance criteria for PCSK9 inhibitor therapy. If 30% of patients with high LDLC and HeFH-CVD are eligible for PCSK9 inhibitors, then specialty pharmaceutical pricing models (~$14,300/year) will collide with an estimated 16–21 million HeFH-CVD patients. Although the costs for PCSK9 inhibitors given to an estimated 16 to 21 million patients are extraordinary ($228–300 billion), we speculate that, when weighed against direct and indirect costs of CVD, on balance, the cost to society might be either none, or that society would, in fact, save money by an anticipated 50% reduction of CVD events with PCSK9 inhibitors. Whether the health care savings arising from the anticipated reduction of CVD on the PCSK9 inhibitors justify the broad population use of these agents remains to be determined.