

Proposed Project Scope

The Effectiveness of PCSK9 Inhibitors in Familial Hypercholesterolemia: Observational Study

July 2024

Background

This query will focus on conducting an observational study that will include the monoclonal antibody inhibitors of *PCSK9*, namely evolocumab and alirocumab, to determine the drugs' benefits in the real world in patients with familial hypercholesterolemia.

Table 1: Policy Questions

Item	Policy question
1	How many patients are achieving a 40% low-density lipoprotein reduction with PCSK9 inhibitors?
2	What are the incidences of nonfatal and fatal cardiovascular events in patients who are prescribed PCSK9 inhibitors in Canada?

Table 2: Products Available in Canada

Product	Manufacturer
alirocumab (Praluent)	Sanofi-Aventis Canada Inc.
evolocumab (Repatha)	Amgen Canada Inc.

Project Description

Table 3: Project Scope

Criteria	Description
Population	Any person exposed to alirocumab or evolocumab with or without other lipid-lowering drugs (i.e., statin and/or ezetimibe)
Outcomes	<ul style="list-style-type: none"> Percent change in lipid level Unadjusted and adjusted incidence of nonfatal and fatal ASCVD events (and related ED visits, hospitalizations, and so forth) in those receiving alirocumab or evolocumab
Subgroup stratification or effect modifiers	<ul style="list-style-type: none"> Comorbidities (risk factors for cardiovascular events) Lipid level at baseline Diagnostic criteria for hypercholesterolemia (e.g., high LDL or non-HDL cholesterol) group lab result if available (e.g., > 190 mg/dL LDL or > 220 non-HDL; ICD codes for hypercholesterolemia; ICD-9/10) Age (< 18 years, 18 to 64 years, > 64 years) Sex and/or gender Lipid-lowering therapy (previous and current use) History of cardiac disease (none, 1 event, multiple events)
Study design	Retrospective cohort study
Setting	AB, BC, ON

AB = Alberta; ASCVD = atherosclerotic cardiovascular disease; BC = British Columbia; ED = emergency department; HDL = high-density lipoprotein; ICD = International Classification of Diseases; LDL = low-density lipoprotein; ON = Ontario.

Table 4: Research Questions

Item	Research question
1	What treatment targets are patients with familial hypercholesterolemia achieving on treatment with alirocumab or evolocumab?
2	What are the cardiovascular event rates with alirocumab and evolocumab?

Status of Document

The proposed project scope document is being posted for feedback.