RATIONALE FOR INCLUSION IN PA PROGRAM

Background
Repatha is used in addition to diet and maximally tolerated statin therapy in adult patients with heterozygous familial hypercholesterolemia (HeFH), homozygous familial hypercholesterolemia (HoFH) or patients with clinical atherosclerotic cardiovascular disease such as heart attacks or strokes, who require additional lowering of LDL cholesterol. HeFH is an inherited condition that causes high levels of low-density lipoprotein (LDL) cholesterol. Repatha provides another treatment option for patients with known cardiovascular disease who have not been able to lower their LDL cholesterol enough on statins. A high level of LDL cholesterol (known as “bad” cholesterol) in the blood is linked to cardiovascular disease. Heart disease is the number one cause of death for Americans, both men and women (1).

Repatha is an antibody that targets a specific protein, called PCSK9, which works by reducing the number of receptors on the liver that remove LDL cholesterol from the blood. By blocking PCSK9’s ability to work, more receptors are available to get rid of LDL cholesterol from the blood and, as a result, lower LDL cholesterol levels (1).

Regulatory Status
FDA Indicated for: Repatha is a PCSK9 (Proprotein Convertase Subtilisin Kexin Type 9) inhibitor antibody indicated as adjunct to diet and maximally tolerated statin therapy for the treatment of: (2)

1. Maximally tolerated statin therapy for treatment of adults with heterozygous familial hypercholesterolemia (HeFH) or clinical atherosclerotic cardiovascular disease (CVD), who requires additional lowering of low density lipoprotein cholesterol (LDL-C).
2. Other LDL-lowering therapies (e.g., statins, ezetimibe, LDL apheresis) in patients with homozygous familial hypercholesterolemia (HoFH) who require additional lowering of LDL-C.

Limitations of Use:
The effect of Repatha on cardiovascular morbidity and mortality has not been determined (2).

The safety and effectiveness of REPATHA have not been established in pediatric patients with HoFH who are younger than 13 years old. The safety and effectiveness of REPATHA have not been established in pediatric patients with primary hyperlipidemia or HeFH (2).

Physicians often measure CK in patients about to begin statins or already on statins. Many
physicians will not start or continue statins to lower LDLC in asymptomatic patients with high CK because of concern about possible statin-induced myositis-rhabdomyolysis. No patients during follow-up on statins developed CK more than 10 times the UNL (2500 IU/L). High pretreatment CK, predominantly 1 to 5 times the UNL, as in the current report, should not be an impediment to start or continue statins to lower LDLC (3).

Summary
Repatha is used in addition to diet and maximally tolerated statin therapy in adult patients with homozygous familial hypercholesterolemia (HoFH), heterozygous familial hypercholesterolemia (HeFH) or patients with clinical atherosclerotic cardiovascular disease such as heart attacks or strokes, who require additional lowering of LDL cholesterol. Repatha is an antibody that targets a specific protein, called PCSK9, which works by reducing the number of receptors on the liver that remove LDL cholesterol from the blood. By blocking PCSK9's ability to work, more receptors are available to get rid of LDL cholesterol from the blood and, as a result, lower LDL cholesterol levels. The safety and efficacy of Repatha in pediatric patients 18 years or less have not been established (1-2).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Repatha while maintaining optimal therapeutic outcomes.

References