VI.2 Elements for a public summary

VI.2.1 Overview of disease epidemiology

Ezetimibe is used to treat hypercholesterolemia (excess cholesterol in the blood stream). It does this by lowering levels of total cholesterol, "bad" cholesterol (LDL cholesterol) and fatty substances called triglycerides in the blood, whilst raising levels of "good" cholesterol (HDL cholesterol). The active ingredient ezetimibe reduces the cholesterol absorbed in your digestive tract. If you have heart disease, ezetimibe combined with cholesterol-lowering medicines called statins reduces the risk of heart attack, stroke, surgery to increase heart blood flow, or hospitalisation for chest pain.

Familial hypercholesterolaemia is a genetic disorder caused by a defect in a gene which controls the way cholesterol is handled in the body. As a result of the defect, bad cholesterol is not broken down properly and builds up in the bloodstream. In most cases the defective gene is inherited from one parent (heterozygous inheritance). If you inherit it from both parents (homozygous inheritance), the condition is more severe. Heterozygous familial hypercholesterolaemia affects about 1 in 500 people in the US and Europe (reference 1). However, familial hypercholesterolemia is more common in other populations. The homozygous condition is rare; one case per million persons in the United States.

Phytosterolaemia (sitosterolaemia) is a very rare inherited sterol storage disease characterized by tendon and tuberous xanthomas and by a strong predisposition to premature coronary atherosclerosis. In addition to increased or normal serum cholesterol, patients are found to have markedly elevated concentrations of the phytosterols sitosterol and campesterol.

VI.2.2 Summary of treatment benefits

Based on the available data from clinical studies and clinical experience of several years, ezetimibe represents an effective drug in the indications listed above.

For the treatment of primary hypercholesterolaemia, the effectiveness of adding ezetimibe to statin therapy was compared to placebo (dummy treatment) in 769 adults. Patients received either ezetimibe 10mg daily or placebo (dummy treatment) for eight weeks. The main outcome measure tested was the lowering of 'bad' cholesterol (LDL-cholesterol) levels. 71.5% of patients treated with ezetimibe and a statin reached their cholesterol level goals compared to 18.9% in the placebo group.

For the treatment of familial hypercholesterolaemia in adolescents, the effectiveness of ezetimibe was compared to placebo in 248 patients. Patients were treated with either ezetimibe 10mg daily or placebo (dummy treatment) for 53 weeks combined with simvastatin. Ezetimibe combined with placebo (dummy treatment) was shown to be more effective in lowering cholesterol levels.

In a double-blind, placebo-controlled, 8-week trial, 37 patients with homozygous sitosterolaemia were randomized to receive Ezetimibe 10mg (n=30) or placebo (n=7). Some patients were receiving other treatments (e.g. statins, resins) Ezetimibe significantly lowered the two major plant sterols. Sitosterol and campesterol, by 21% and 24% from baseline, respectively. The effects of decreasing sitosterol on morbidity and mortality in the population are not known.