EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Press Office

Press release

First-in-class treatment to lower cholesterol

Repatha to offer therapy for patients unable to control high cholesterol with currently available treatment

The European Medicines Agency (EMA) has recommended authorising Repatha (evolocumab) as treatment to lower high levels of cholesterol in the blood of people who are unable to control their cholesterol despite taking optimal doses of statins or who cannot take statins. It should be used in addition to a healthy diet. Other lipid-lowering therapies (statins and others) should also be used if tolerated. Repatha is also indicated to treat people with homozygous familial hypercholesterolaemia, a rare inherited disorder in which levels of LDL-cholesterol ('bad cholesterol') are higher than normal from birth. It is intended for injection under the skin either once every two weeks, or once a month.

High levels of cholesterol in the blood are common risk factors for heart disease, which is the leading cause of death globally.

Repatha is the first monoclonal antibody (a type of protein) in this therapeutic area and provides a new treatment option for patients who are unable to control their high cholesterol despite taking currently available therapies. Repatha blocks the PCSK9 protein, which would otherwise lower the number of LDL-receptors in the liver and through this, diminishes its ability to remove LDL-cholesterol from the blood.

The efficacy of Repatha as a lipid-lowering agent was assessed in nine trials (about 5,500 people) in patients with hypercholesterolaemia and mixed dyslipidaemia, and in two studies (about 250 people) in patients with homozygous familial hypercholesterolaemia. Repatha reduced LDL-cholesterol for both patient groups. Available evidence does not yet allow the longer term benefits of Repatha for patients in reducing heart disease or death from heart disease to be determined.

The Committee for Medicinal Products for Human Use (CHMP) also looked at safety information from patients with hypercholesterolaemia and mixed dyslipidaemia (over 6,000 patients followed for at least six months and over 1,100 patients followed for at least two years). The Committee considered that the safety profile of Repatha is acceptable, with few patients discontinuing treatment or showing serious adverse events. A similar safety profile was observed in patients with homozygous familial hypercholesterolaemia. Further data will be collected to assess the implications of very low cholesterol levels.

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The full indication for Repatha approved by the CHMP is as follows:

- Repatha is indicated in adults with primary hypercholesterolaemia (heterozygous familial and non-familial) or mixed dyslipidaemia, as an adjunct to diet:
    - in combination with a statin or statin with other lipid-lowering therapies in patients unable to reach LDL-C goals with the maximum tolerated dose of a statin; or
    - alone or in combination with other lipid-lowering therapies in patients who are statin-intolerant, or for whom a statin is contra-indicated;
- Repatha is indicated in adults and adolescents aged 12 years and over with homozygous familial hypercholesterolaemia in combination with other lipid-lowering therapies;
- The effect of Repatha on cardiovascular morbidity and mortality has not yet been determined.

The company received scientific advice on clinical aspects of the application from the CHMP. This is one of the Agency's main tools to facilitate and stimulate research and development within the European Union (EU).

The opinion adopted by the CHMP at its May 2015 meeting is an intermediary step on Repatha's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, each Member State will take a decision on price and reimbursement based on the potential role/use of this medicine in the context of its national health system.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The marketing authorisation applicant for Repatha is Amgen Europe B.V.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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