30 May 2013
EMA/327261/2013
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Lojuxta

lomitapide

On 30 May 2013 the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Lojuxta, 5mg, 10mg, 20mg hard capsules intended as an adjunct to a low fat diet and other lipid lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adult patients with homozygous familial hypercholesterolaemia (HoFH). The applicant for this medicinal product is Aegerion Pharmaceuticals. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Lojuxta is lomitapide, other lipid modifying agents, plain (ATC code: C10AX12) and is a selective inhibitor of microsomal transfer protein (MTP), an intracellular lipid-transfer protein responsible for binding and shuttling lipids between membranes. MTP plays a key role in the assembly of apo B containing lipoproteins in the liver and intestines. Inhibition of MTP reduces lipoprotein secretion and circulating concentrations of lipoprotein-borne lipids including cholesterol and triglycerides.

The benefits with Lojuxta are its ability to consistently reduce LDL-cholesterol levels by approximately 40% in HoFH patients. The most common side effects are gastrointestinal and hepatic adverse events, is some cases leading to treatment discontinuation; weight loss has also been observed.

A pharmacovigilance plan for Lojuxta will be implemented as part of the marketing authorisation.

The approved indication is:

"*Lojuxta is indicated as an adjunct to a low-fat diet and other lipid-lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adult patients with homozygous familial hypercholesterolaemia (HoFH).*

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7<sector fax>
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

Genetic confirmation of HoFH should be obtained whenever possible. Other forms of primary hyperlipoproteinemia and secondary causes of hypercholesterolemia (e.g., nephrotic syndrome, hypothyroidism) must be excluded."

It is proposed that treatment with Lojuxta should be initiated and monitored by a physician experienced in the treatment of lipid disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit-to-risk balance for Lojuxta and therefore recommends the granting of the marketing authorisation under exceptional circumstances.