HERTFORDSHIRE MEDICINES MANAGEMENT COMMITTEE (HMMC)

LOMITAPIDE FOR HOMOZYGOUS FAMILIAL HYPERCHOLESTEROLAEMIA (HoFH)

NOT RECOMMENDED

<table>
<thead>
<tr>
<th>Name: generic (trade)</th>
<th>What it is</th>
<th>Indication</th>
<th>Date decision last revised</th>
<th>Decision status</th>
<th>NICE / SMC Guidance</th>
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<tr>
<td>Lomitapide (Lojuxta®)</td>
<td>Selective inhibitor of microsomal transfer protein, reducing lipoprotein secretion</td>
<td>Adjunctive therapy to a low-fat diet and other lipid-lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adults with HoFH.</td>
<td>April 2014</td>
<td>Interim – NHS England commissioning responsibility from April 2015</td>
<td>NICE - None SMC - None</td>
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</tbody>
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Lomitapide is the commissioning responsibility of CCGs for the financial year 2014-15, and will be the commissioning responsibility of NHS England from April 2015.

HMMC Recommendation:
Prescribing **NOT RECOMMENDED** within Hertfordshire in primary, secondary or tertiary care.

**Efficacy**
- In a phase III trial, 29 patients with HoFH experienced a mean reduction in LDL-C of 50% by week 26.
- Effect on cardiovascular event rate is unknown.

**Safety**
- In the phase III trial, gastrointestinal symptoms were experienced by 80% of patients. There was an increase in mean hepatic fat, from 1% at baseline to 8.6% at week 26. 4 patients had liver enzyme elevations between 5 and 11 times the upper limit of normal.
- Long term safety is unknown – safety studied up to week 78 in trial situation.

**Cost**
- Lomitapide, as 20-60mg/day, costs £230,000-£695,000/patient/year
- There is no information on cost effectiveness.

**Patient Factors**
- Current oral drug treatments options are of limited efficacy for HoFH. Patients may prefer a more effective oral treatment instead of receiving LDL-C plasma apheresis weekly or fortnightly, however patients may still need to receive LDL-C apheresis in addition to lomitapide.

This HMMC recommendation is based upon the evidence available at the time of publication. The recommendation will be reviewed upon request in the light of new evidence becoming available.
Assessment against Ethical Framework

Evidence of Clinical Effectiveness and Safety
- Main evidence of efficacy is from a phase III trial in 29 patients with HoFH, who experienced a mean reduction in LDL-C of 50% by week 26.
- No information on cardiovascular outcomes is available.
- Main side effects are gastrointestinal, experienced by 80% of patients in clinical trial.
- Raised liver enzymes were raised in 4 trial patients, between 5 and 11 times the upper limit of normal.
- In trial patients, mean hepatic fat increased from 1% at baseline to 8.6% at week 26.
- Long term safety is unknown. Ongoing safety study was up to week 78 in trial population.

Cost of treatment and cost effectiveness
- Cost is between £230,000-£695,000/patient/year, depending upon the dose (maintenance dose 20-60mg/day).
- There is no information on cost effectiveness.

The needs of the population
The needs of the population appear to be high as this is a severe disease with significant morbidity and mortality. Current drug treatment options are not very effective, and the alternative treatment is LDL-C apheresis weekly or fortnightly. The incidence of HoFH is approximately one in a million.

The needs of the community
Use of lomitapide may have a significant effect on the local health economies’ ability to fund other treatments.

Equity
No impact anticipated.

Policy drivers
NHS England has advised that it will become the responsible commissioner for lomitapide from 2015-16 financial year. For 2014-15, NHS England has advised that responsibility for commissioning decision lies with CCGs. A positive CCG decision in 2014-15 may set a precedent for NHS England commissioning decision.

Implementability
No issues identified.

References
- eMC Summary of Product Characteristics, accessed 25.03.2014
  http://www.medicines.org.uk/emc/medicine/28513
- eMC Dictionary of Medicines and Devices Browser, accessed 25.03.2014
- Lomitapide for homozygous familial hypercholesterolaemia (Comment), The Lancet, Vol 381, January 5 2013, pgs 7-8

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