

Treatment Appraisal: Decision Summary

Date	13 th July 2010
Appraisal	Amifampridine phosphate (Firdapse®▼) for Lambert-Eaton myasthenic syndrome (LEMS)
Details	NETAG was requested by a member organisation to conduct an appraisal of and issue a recommendation regarding amifampridine phosphate (Firdapse®) for the treatment of Lambert-Eaton myasthenic syndrome.
Recommendation	<p>Amifampridine phosphate (Firdapse®) for Lambert-Eaton myasthenic syndrome is not recommended for use within NHS North East.</p> <p>The group did not consider amifampridine phosphate (Firdapse®) to be a cost-effective treatment contingent on the continued availability of unlicensed amifampridine (3,4-diaminopyridine).</p>
Clinical evidence summary	<p>The group observed that no additional clinical evidence in LEMS patients was produced to support the license application for Firdapse® and that all clinical evidence originated from studies of amifampridine base.</p> <p>The group was mindful of guidance from professional and regulatory authorities regarding the use of unlicensed medicines and the legal implications of their prescribing. The group also noted the apparent absence of issues arising from extensive experience of using unlicensed amifampridine.</p>
Cost analysis summary	The group noted that Firdapse® is currently substantially more costly than equivalent doses of unlicensed amifampridine. The group noted the additional patient support and research programme that the manufacturer of Firdapse® has committed to. However, it was considered that the incremental cost of Firdapse® does not represent value for NHS North East.
Financial impact	<ul style="list-style-type: none"> • It is estimated that the prevalence of LEMS is about five to eight patients within NHS North East. • The annual cost of treatment with Firdapse® is estimated to be between £30,000 to £45,000 per patient based on typical doses. • The annual cost of treatment with equivalent doses of amifampridine base is currently estimated at £1,500 to £2,200 per patient. • The total incremental cost to NHS North East of using Firdapse® instead of amifampridine base is estimated at between £147,000 and £236,000 per annum based on current prices of amifampridine and a mean dose of 40 mg daily.
PbR: Excluded	
Further research or information	The group is mindful of the availability of amifampridine base (3,4-diaminopyridine) and will review this recommendation if adverse factors relating to its provision arise.

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