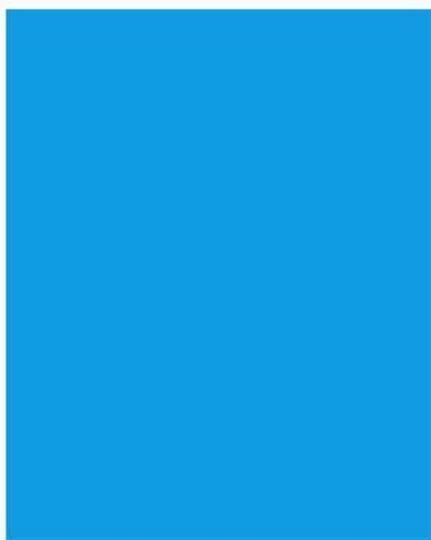


**Clinical Commissioning
Policy Statement:
Amifampridine (Firdapse) for
Lambert Easton Myasthenic
Syndrome (LEMS)**

April 2013

Reference : NHSCB/D04/PS/a



NHS Commissioning Board Clinical Commissioning Policy Statement: Amifampridine (Firdapse) for Lambert Easton Myasthenic Syndrome (LEMS)

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**Prepared by the NHS Commissioning Board Clinical Reference Group for
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<p>POLICY STATEMENT:</p> <p>Amifampridine (Firdapse®)</p>	<p>Policy Ref:</p> <p>NHSCB/D04/PS/a</p>
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Treatment:	<p>Amifampridine is 3, 4 Diaminopyridine (phosphate form) (Firdapse®). It has recently been given market authorisation under the European Orphan Drugs legislation.</p> <p>BioMarin UK Ltd.</p>
For:	<p>Symptomatic treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). It is licensed for use in LEMS patients only and to a maximum daily dose of 60mg.</p>
Background:	<p>LEMS is a chronic progressive debilitating condition of presynaptic neuromuscular transmission. It is caused by insufficient release of a chemical neuro transmitter called acetylcholine from the synaptic vesicles resulting in impaired nerve signal transmission. In 75 to 95% of cases the etiology can be traced to auto-antibodies that are directed against voltage gated calcium channels.</p> <p>The symptoms of LEMS vary in severity but are characterised by muscle weakness and excessive fatigue, (particularly of the legs and trunk), drooping eyelids and speech impairment. Sensory disturbances such as numbness or tingling are also common. Other features include dry eyes, dry mouth, constipation, and impaired sweating amongst other symptoms. The onset of symptoms is gradual and insidious.</p> <p>LEMS is strongly associated with cancer, especially small-cell lung cancer (SCLC). It is estimated that about 3% of patients with SCLC have LEMS, and 40 to 60% of patients with LEMS have SCLC and 5% have other cancers. Where LEMS occurs in the absence of cancer it is often associated with an autoimmune disorder.</p> <p>LEMS is a rare condition with prevalence estimated at 5 per 2 million. It is therefore estimated that there are 150 patients with LEMS in the UK. Annual incidence is estimated at 1 per 2.5 million of the population.</p>
Commissioning position:	<p>Amifampridine (Firdapse®) will not be routinely commissioned for the treatment of Lambert-Eaton Myasthenic Syndrome.</p>

Effective from: 1 April 2013

Evidence summary: The Scottish Medicines Consortium (SMC) published their advice on amifampridine phosphate (Firdapse®) in 2012.

It is not recommended for use in NHS Scotland.

The SMC advice states:

There are no clinical data for amifampridine phosphate and efficacy has been extrapolated from studies of amifampridine base (3,4-diaminopyridine), to which amifampridine phosphate has been accepted to be bioequivalent by the European Medicines Agency. In randomised controlled studies in patients with LEMS, 3,4-diaminopyridine treatment was associated with greater improvement in muscle strength and neuromuscular transmission than placebo.

The submitting company's justification of the treatment's cost in relation to its health benefits was not sufficient and in addition, the company did not present a sufficiently robust economic analysis to gain acceptance by SMC.¹

Further details of the economic analyses are available in the SMC report.¹ This includes estimates of cost per quality adjusted life year (QALY) for this treatment which, at £92,267, fall outside the range considered acceptable for use of a treatment in NHS England.

Equality impact: The NHS CB has a duty to have regard to the need to reduce health inequalities in access to health services and health outcomes achieved as enshrined in the Health and Social Care Act 2012. The NHS CB is committed to ensuring equality of access and non-discrimination, irrespective of age, gender, disability (including learning disability), gender reassignment, marriage and civil partnership, pregnancy and maternity, race, religion or belief, sex (gender) or sexual orientation. In carrying out its functions, the NHS CB will have due regard to the different needs of protected equality groups, in line with the Equality Act 2010. This document is compliant with the NHS Constitution and the Human Rights Act 1998. This applies to all activities for which they are responsible, including policy development, review and implementation.

Responsible CRG: Neurosciences CRG

Date approved by NHSCB Board: 1 April 2013

Policy review date: 2013/14

Version: 1

References

Scottish Medicines Consortium. Amifampridine 10mg tablet, as phosphate (Firdapse®) SMC No.(660/10). July 2012. Available from: [http://www.scottishmedicines.org.uk/files/advice/amifampridine_phosphate_FIRDAPSE_FINAL_July_2012_for_website.pdf](http://www.scottishmedicines.org.uk/files/advice/amifampridine_phosphate_Firdapse_FINAL_July_2012_for_website.pdf) . Accessed 54/12/12.

Change Notice for Published Specifications and Products developed by Clinical Reference Groups (CRG)

Amendment to the Published Products

Product Name	Amifampridine (Firdapse) for Lambert Easton Myasthenic Syndrome (LEMS)
Ref No	D04/PS/a
CRG Lead	Neurosciences

Description of changes required

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Changes made by	Date change made
Amifampridine (Firdapse)	Amifampridine (Firdapse) for Lambert Easton Myasthenic Syndrome (LEMS)	Title page and 2 nd and 3 rd pages	To standardise naming and coding of products	Programme of Care Director for Trauma	September 2013