NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Masitinib for treating amyotrophic lateral sclerosis

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of masitinib within its marketing authorisation for treating amyotrophic lateral sclerosis.

Background

Amyotrophic lateral sclerosis is a neurodegenerative condition that affects the brain and spinal cord. It is characterised by the degeneration of motor neurones, leading to muscle weakness. Initial symptoms vary and may include muscle weakness, wasting, cramps and stiffness of arms and/or legs, problems with speech and/or swallowing or, more rarely, breathing problems[3]. In the UK, amyotrophic lateral sclerosis is sometimes called motor neurone disease.

Between 3200 and 4000 people in England have amyotrophic lateral sclerosis[4]. It can affect adults at any age, but most people are diagnosed over the age of 50. About 90% of people do not have a family history of the disease (known as sporadic disease). About 5-10% of people do have a family history of the disease (known as familial). The sporadic and familial forms of the disease are treated in the same way. Amyotrophic lateral sclerosis is more common in men than in women[5].

There is currently no cure for amyotrophic lateral sclerosis. NICE technology appraisal 21 recommends riluzole for treating amyotrophic lateral sclerosis. NICE guideline 42 on the assessment and management of motor neurone disease recommends care by a multidisciplinary team including, where appropriate:

- Psychological and social care support.
- Interventions to manage symptoms, for example pharmacological treatment for muscle problems.
- Equipment to aid activities of daily living and mobility.
- Support for nutrition, communication, and respiratory function including surgical interventions if necessary (for example, to enable feeding).

The technology

Masitinib (brand name unknown, AB Science) is a tyrosine kinase inhibitor that targets mast cells and macrophages. It is proposed to inhibit inflammatory

processes involved in some central nervous system diseases. It is administered orally.

Masitinib does not currently have a marketing authorisation in the UK for treating amyotrophic lateral sclerosis. It has been studied in clinical trials that compared masitinib plus riluzole with placebo plus riluzole in adults with familial or sporadic amyotrophic lateral sclerosis.

Intervention(s)	Masitinib in combination with riluzole
Population(s)	People with amyotrophic lateral sclerosis
Comparators	• Riluzole and best supportive care • Best supportive care
Outcomes	The outcome measures to be considered include: • overall survival • disease progression • forced vital capacity (FVC) • time to first tracheotomy • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations and NICE Pathways	Related Technology Appraisals: Technology Appraisal No. 20, January 2001, 'Motor neurone disease – riluzole.' Moved to static list, April 2006.

	Related Guidelines: NICE guideline 42, February 2016, 'Motor neurone disease: assessment and management'. Related Quality Standards: 'Motor Neurone Disease' 2015 NICE Quality Standard. Publication expected August 2016 Related Interventional Procedure guidance: 'Intramuscular diaphragm stimulation for ventilator-dependent chronic respiratory failure due to neurological disease' July 2009 NICE Interventional Procedures Guidance 307. Related NICE Pathways: 'Motor Neurone Disease' (2010) NICE pathway http://pathways.nice.org.uk/pathways/motor-neurone-disease
Related National Policy	NHS England (2016) Manual for prescribed specialised services, section 11. Adult specialist neurosciences services and section 134. Specialist services to support patients with complex physical disabilities (all ages) https://www.england.nhs.uk/commissioning/wp-content/uploads/sites/12/2016/06/pss-manual-may16.pdf NHS England (2013) NHS standard contract for neurosciences specialised neurology (adult). https://www.england.nhs.uk/wp-content/uploads/2013/06/d04-neurosci-spec-neuro.pdf Department of Health, NHS Outcomes Framework 2015-2016, Dec 2014. Domains 1, 2, 4 and 5. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/385749/NHS_Outcomes_Framework.pdf

References

1. Motor Neurone Disease Association (2015) Motor neurone disease: a guide for GP's and primary care teams. Accessed March 2016

2. NICE (2016) Motor neurone disease: assessment and management. NICE clinical guideline 42. Accessed March 2016