NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Masitinib with riluzole for treating amyotrophic lateral sclerosis ID6257

Draft scope

Draft remit/evaluation objective

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

Background

Amyotrophic lateral sclerosis (ALS) is the most common type of motor neurone disease (MND). It is a neurodegenerative condition that affects the brain and spinal cord and is characterised by the degeneration of motor neurones, leading to muscle weakness. Initial symptoms of ALS 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[1]. According to the literature about 5-10% of people with ALS have a family history of the disease (known as familial ALS) and about 90% do not (known as sporadic disease)[2]

Prevalence estimates are uncertain, but a maximum of 4000 people in England and Wales are thought to have MND and 90% of these people have the ALS type of the disease. [3,4,5] It can affect adults of any age, but most people are diagnosed between the ages of 50 and 70.[6] ALS is more common in men than women.[6] Approximately 1,500 people are diagnosed with ALS per year in the UK[7] and average life expectancy is usually two to five years from the onset of symptoms.[8]

There is currently no cure for ALS. NICE technology appraisal 20 recommends riluzole for treating ALS. 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) in combination with riluzole does not currently have a marketing authorisation in the UK for treating ALS. It has been studied in combination with riluzole in clinical trials compared with placebo for treating familiar or sporadic ALS in adults.

Intervention(s)	Masitinib in combination with riluzole
Population(s)	Adults with amyotrophic lateral sclerosis
Subgroups	If the evidence allows the following subgroups will be considered: • Choice of background therapy • Stage/severity of the disease
Comparators	Established clinical management without masitinib including but not limited to: • Riluzole • Tofersen (subject to ongoing NICE appraisal) • Best supportive care
Outcomes	The outcome measures to be considered include: • overall survival • disease progression • time to permanent ventilation (tracheostomy or more than 22 hours a day of non-invasive ventilation) • respiratory status • nutritional status • 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. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account. The availability and cost of biosimilar and genetic products should be taken into account.

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	**Related Technology Appraisals:** 'Guidance on the use of Riluzole for the treatment of Motor Neurone Disease' (2001). NICE Technology Appraisal 20. Guidance on static list. **Appraisals in development (including suspended appraisals)** 'Masitinib for treating amyotrophic lateral sclerosis' NICE technology appraisals guidance [ID967]. Publication date to be confirmed [suspended] **Related Guidelines:** 'Motor Neurone Disease: assessment and management' (2016). NICE guideline 42. Last updated July 2019. **Related Interventional Procedures:** 'Intramuscular diaphragm stimulation for ventilator-dependent chronic respiratory failure caused by motor neurone disease' (2017) Interventional procedures guidance 593 **Related Quality Standards:** 'Motor Neurone Disease' (2016) NICE quality standard 126
Related National Policy	The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018) Manual for prescribed specialised services 2018/19 Chapter 11: Adult specialist neurosciences services Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 1, 2, 4, 5. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017 NHS RightCare (2019) RightCare Progressive Neurological Conditions Toolkit

Questions for consultation

Is the population defined appropriately?

Which treatments are considered to be established clinical practice in the NHS for amyotrophic lateral sclerosis?

Where do you consider masitinib in combination with riluzole will fit into the existing care pathway for amyotrophic lateral sclerosis?

Would masitinib be used as an add-on treatment to riluzole if the use of riluzole has been initiated?

Have all relevant comparators for masitinib in combination with riluzole been included in the scope?

What constitutes best supportive care in ALS in the NHS?

Are the outcomes listed appropriate?

Are the subgroups listed appropriate? Is "choice of background therapy" an appropriate subgroup? Would masitinib in combination with riluzole be a candidate for managed access?

Do you consider that the use of masitinib in combination with riluzole can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which masitinib in combination with riluzole will be licensed

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access masitinib in combination with riluzole;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-tehnology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. Motor neurone disease association (2019) Basic facts about MND. Accessed April 2023
2. Tang L, Ma Y, Liu X-l, Chen L, Fan D-s (2019). Better survival in female SOD1-mutant patients with ALS: a study of SOD1-related natural history. Translational Neurodegeneration. 8(1):2. Accessed April 2023

3. Brown CA, et al (2021) Estimated Prevalence and Incidence of Amyotrophic Lateral Sclerosis and SOD1 and C9orf72 Genetic Variants. Neuroepidemiology 55(5):342-353.
4. National Institute for Health and Care Excellence (2016). Motor neurone disease: assessment and management. NICE guideline NG42. Accessed August 2021.
5. Sheffield MND Care and Research Centre (2015) What is the difference between MND and ALS? Accessed April 2023.
6. An introduction to motor neurone disease (2019) Motor Neurone Disease Association. Accessed April 2023
7. Gowland A et al. (2019). Predicting the future of ALS: the impact of demographic change and potential new treatments on the prevalence of ALS in the United Kingdom, 2020-2116. Amyotrophic Lateral Sclerosis Frontotemporal Degeneration. 20(3-4):264-274.
8. What is Motor Neurone Disease (2022) Motor Neurone Disease Association. Accessed April 2023