

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

SCOPE

1 **Guideline title**

Motor neurone disease: the assessment and management of motor neurone disease

1.1 **Short title**

Motor neurone disease

2 **The remit**

The Department of Health has asked NICE: 'to develop a clinical guideline on the assessment and management of motor neurone disease'.

3 **Need for the guideline**

3.1 **Epidemiology**

- a) Motor neurone disease (MND) is a neurodegenerative condition which affects the brain and spinal cord and is primarily characterised by degeneration of the motor neurones and subsequent loss of motor neurone function.
- b) MND presents in 4 main forms;
 - I. Amyotrophic lateral sclerosis (ALS) which results in upper and lower motor neurone damage with symptoms of wasting and weakness. This occurs in approximately two-thirds of people diagnosed with MND.
 - II. Progressive bulbar palsy, which is caused by lower motor neurone damage in the head and neck, leading to difficulties

with swallowing and speech. Progressive bulbar palsy occurs in a quarter of people with MND.

- III. Progressive muscular atrophy, which is due to lower motor nerve loss, resulting in weakness and wasting of muscles, especially in the arms. It affects 1 in 10 people with MND.
 - IV. Primary lateral sclerosis, in which upper motor neurone nerve loss causes increasing stiffness. With this slowly progressive form the prognosis is usually longer – an average of 10–15 years in total.
- c) Although MND may initially present in one of the above forms, as the disease progresses the effects on each person vary as both lower and upper motor neurones can become affected in any part of the body. Most people lose the ability to walk, move their arms, swallow, speak and have difficulty breathing, eventually leading to death. Approximately 50% of people with MND show cognitive change, varying from mild frontal lobe changes, which may affect decision making, to frontal temporal dementia.
 - d) MND is thought to develop as a result of a complex interplay between genetic, environmental and lifestyle factors in the ageing brain.
 - e) The majority of people with MND do not have a family history of the disease (known as ‘sporadic’ MND), but approximately 5–10% have a close family relative who has the disease (‘familial’ MND). Of those who have familial MND, a large proportion have a mutation in the *C9orf72* gene and the other genes and proteins that are linked to familial MND, including *SOD1*, *TARDBP-43* and *FUS*.
 - f) It is estimated that there are up to 5,000 people with MND in the UK. Approximately 1,100 people are diagnosed annually in the UK, a diagnosis which has a wide-ranging impact not only on the person themselves but on their family and friends.

- g) The onset of disease occurs predominantly between the ages of 55 and 65 years, affecting slightly more men than women. MND is severely life-shortening, with 50% of people dying from respiratory failure within 3 years of developing their first symptoms. However, some people have a slower disease course and may survive for 10 years or more.

3.2 Current practice

- a) Motor neurone disease is incurable and the management of symptoms varies across England and Wales. In addition to providing care, the multidisciplinary team may enable people with MND to make treatment preferences in advance, in case their preferences cannot be communicated at the advanced or end stages of the disease. Decisions at the early stages can include where treatment is to be given (at home or in a care home, hospice or hospital) and which medications or interventions should be given as the disease progresses.
- b) Drugs can be used for symptom management, but riluzole is the only pharmacological drug licensed in the UK to slow the progression of MND. Other medications and interventions can be used to manage symptoms. For people who have difficulty breathing, non-invasive ventilation may be delivered through a nasal mask, oronasa mask or mouthpiece.

4 The guideline

The guideline development process is described in detail on the NICE website (see section 6, 'Further information').

This scope defines what the guideline will (and will not) examine, and what the guideline developers will consider. The scope is based on the referral from the Department of Health.

The areas that will be addressed by the guideline are described in the following sections.

4.1 Population

4.1.1 Groups that will be covered

- a) Adults (aged 18 and over) with MND.
- b) People with frontal temporal dementia will be considered as a separate patient subgroup.

4.1.2 Groups that will not be covered

- a) Children and young people (under 18 years).
- b) Adults with other neurodegenerative disorders who do not have MND.
- c) People diagnosed with Kennedy's disease.

4.2 Healthcare setting

- a) All settings in which NHS care is provided.

4.3 Management

4.3.1 Key issues that will be covered

- a) Timeliness of diagnosis, and communicating with patients and their families and carers about the diagnosis.
- b) Communicating with patients and their families and carers about prognosis and ongoing care.
- c) Monitoring and ongoing assessment, including:
 - assessment of cognitive impairment
 - coordination of care and support across health and social care
 - disease progression.
- d) Symptom management, including:
 - muscle stiffness and cramp

- muscle weakness
 - communication problems
 - swallowing difficulties and secretion management, including drooling
 - nutrition, including weight management and timing of gastrostomy
 - breathing difficulties, including cough assistance.
- e) Psychosocial support for people with MND and their families and carers.
- f) Identification of social care needs for people with MND and their carers.
- g) Managing discontinuation of non-invasive ventilation.
- h) Preparation for, and anticipation of, end of life.

4.3.2 Issues that will not be covered

- i) Diagnosis, including investigations.
- j) Complementary therapies.
- k) Riluzole.
- l) Tracheostomy.
- m) Dietary supplements with the aim of modifying disease progression.
- n) Enteral feeding.

4.4 Main outcomes

- o) Health-related quality of life.
- p) Patient and carer-reported outcomes, for example, symptoms, satisfaction and pain.
- q) Function measured by disability scores.

- r) Hospital admissions (including unplanned admissions).
- s) Mobility.
- t) Survival.

4.5 Review questions

Review questions guide a systematic review of the literature. They address only the key issues covered in the scope, and usually relate to interventions, diagnosis, prognosis, service delivery or patient experience. Please note that these review questions are draft versions and will be finalised with the Guideline Development Group.

4.5.1 Timeliness of diagnosis

What is the time to diagnosis for people with MND in the UK and what are the main causes of delay?

4.5.2 Communicating with people with motor neurone disease and their families and carers

- a) What are the best methods of communicating diagnosis to people with MND and their families and carers?
- b) What are the best methods of communicating prognosis and choices of ongoing care to people with MND and their families and carers?

4.5.3 Monitoring and ongoing assessment

- a) What are the most clinically and cost effective cognitive assessment tools for people with MND?
- b) Does multidisciplinary team care, including social care, improve patient and carer outcomes in people with MND?
- c) What are the most clinical and cost effective assessment tools for monitoring the progression of MND and what should be the frequency of assessment?

- d) What are the most effective and cost effective assessment tools for assessing the social care needs for people with MND and how often should these needs be reassessed?

4.5.4 Symptom management

- a) What is the clinical and cost effectiveness of pharmacological treatments for managing muscle stiffness in people with MND?
- b) What is the clinical and cost effectiveness of non-pharmacological treatments for managing muscle stiffness in people with MND?
- c) What is the clinical and cost effectiveness of equipment for managing muscle stiffness in people with MND?
- d) What is the clinical and cost effectiveness of pharmacological treatments for managing muscle cramps in people with MND?
- e) What is the clinical and cost effectiveness of non-pharmacological treatments for managing muscle cramps in people with MND?
- f) What is the clinical and cost effectiveness of pharmacological treatments for managing muscle weakness in people with MND?
- g) What is the clinical and cost effectiveness of non-pharmacological treatments for managing muscle weakness in people with MND?
- h) What is the clinical and cost effectiveness of equipment for managing muscle weakness in people with MND?
- i) What is the clinical and cost effectiveness of systems for managing communication problems in people with MND?
- j) What is the clinical and cost effectiveness of pharmacological treatments for secretion management in people with MND?
- k) What is the clinical and cost effectiveness of non-pharmacological treatments for secretion management in people with MND?

- l) What is the clinical and cost effectiveness of treatments for managing swallowing difficulties in people with MND?
- m) What is the clinical and cost effectiveness of treatments for weight management in people with MND?
- n) What is the optimum timing of gastrostomy in people with MND?
- o) What is the clinical and cost effectiveness of cough-assistance techniques for managing respiratory difficulties in people with MND?
- p) What is the clinical and cost effectiveness of pharmacological treatments for managing breathing difficulties in people with MND?

4.5.5 Psychosocial and social support for people with motor neurone disease and their families and carers

- a) What are the psychosocial and social care support needs of people with MND and their families and carers?

4.5.6 Preparation for, and anticipation of, end of life

- a) How should the discontinuation of NIV be managed?
- b) What are the best methods of communication and support to help people with MND and their families and carers prepare for, and anticipate, end of life?

4.6 *Economic aspects*

Developers will take into account both clinical and cost effectiveness when making recommendations involving a choice between alternative interventions. A review of the economic evidence will be conducted and analyses will be carried out as appropriate. The preferred unit of effectiveness is the quality-adjusted life year (QALY), and the costs considered will usually be only from an NHS and personal social services (PSS) perspective. Further detail on the methods can be found in 'The guidelines manual' (see 'Further information').

4.7 Status

4.7.1 Scope

This is the final version of the scope.

4.7.2 Timing

The development of the guideline recommendations will begin in March 2014.

5 Related NICE guidance

5.1 Published guidance

5.1.1 NICE guidance to be updated

This guideline does not intend to update any existing NICE guidance.

5.1.2 NICE guidance to be amalgamated

[Motor neurone disease: the use of non-invasive ventilation in the management of motor neurone disease](#). (NICE clinical guideline 105 (July 2010)).

5.1.3 Other related NICE guidance

- [Opioids in palliative care](#). NICE clinical guideline 140 (2012).
- [Infection control](#). NICE clinical guideline 139 (2012).
- [Patient experience in adult NHS services](#). NICE clinical guideline 138 (2012).
- [End of life care for adults](#). NICE quality standard 13 (2011).
- [Depression with a chronic physical health problem](#). NICE clinical guideline 91(2009).
- [Functional electrical stimulation for drop foot of central neurological origin](#). NICE interventional procedure guidance 278 (2009).
- [Nutrition support in adults](#). NICE clinical guideline 32 (2006).
- [Riluzole \(rilutek\) for the treatment of motor neurone disease](#). NICE technology appraisal guidance 20 (2001).

- [End of life care for infants, children and young people](#) NICE Clinical Guideline (publication date tbc)

5.2 Guidance under development

NICE is currently developing the following related guidance (details available from the NICE website):

- Pressure ulcers. NICE clinical guideline. Published April 2014.
- Multiple sclerosis. NICE clinical guideline. Published October 2014.
- Medicines optimisation. NICE clinical guideline. Publication to be confirmed.
- Homecare. NICE social care guidance. Publication expected 2015.
- Transition between health and social care. Publication expected 2015.

6 Further information

Information on the guideline development process is provided in the following documents, available from the NICE website:

- [‘How NICE clinical guidelines are developed: an overview for stakeholders the public and the NHS’](#)
- [‘The guidelines manual’](#).

Information on the progress of the guideline will also be available from the [NICE website](#).