motor neurone disease association

Response to the consultation on early access to medicines in the UK

Introduction

i. Few conditions are as devastating as motor neurone disease (MND). It is rapidly progressive in the majority of cases, and is always fatal. People with MND will, in varying sequences and combinations, lose the ability to speak, swallow and use their limbs; the most common cause of death is respiratory failure. Most commonly the individual will remain mentally alert as they become trapped within a failing body, although some experience dementia or cognitive change. There are about 5,000 people living with MND in the UK. Half of people with the disease die within 14 months of diagnosis. There is no cure.

ii. The MND Association is the only national organisation supporting people affected by MND in England, Wales and Northern Ireland, with approximately 90 volunteer led branches and 3,000 volunteers. The MND Association's vision is of a World Free of MND. Until that time we will do everything we can to enable everyone with MND to receive the best care, achieve the highest quality of life possible and to die with dignity.

iii. There is currently only one drug that is known to slow the progression of MND, riluzole, and no new drug with this effect is known to be in the pipeline – indeed, as the only drug that slows the progression of any age-related neurodegenerative disease, even riluzole's existence is somewhat remarkable. One new drug, nueudexta, is currently going through the approvals process: it does not slow MND, but addresses emotional lability (uncontrollable laughing or crying), one of the most distressing symptoms of MND.

iv. The limited scope of these proposals, and the modest immediate prospects of a drug-based breakthrough for MND, therefore mean that they are unlikely to benefit people with MND in the medium term. We are in favour, however, of anything that helps people with MND obtain safe and effective treatments more quickly and we hope that people with MND might benefit from these arrangements at some point in the future.

v. We comment only on selected questions below.

Question 1: Do you consider that a scheme that makes available in the UK certain new medicines before they are granted a marketing authorisation (licence) will be of value to patients?

Yes. Given the rapidity of MND in many cases, a delay of twelve months during the authorisation process can be very significant indeed, so there is no doubt that many people with MND would benefit from the proposal if a suitable drug were to be developed.

Question 2: Do you have views about the scope of the proposed scheme (for example the type of illnesses and conditions that will be included)?

MND certainly counts as a life threatening condition with inadequate treatment options, so we are pleased that it is in scope and agree that this is an appropriate focus for the scheme.

One aspect of the scope that we would challenge, however, is the scheme's restriction to medicines. The most effective steps that can be taken to improve both the survival of people with MND and their quality of life, at present, are interventions to support their respiratory function. Significant developments have taken place in this area in recent years. Non-invasive ventilation has been demonstrated to have beneficial effects, but received NICE guidance only after a delay of several years; and a trial is currently underway to investigate the effects of diaphragm pacing for people with MND.

Could the scheme be widened to include interventions of this sort, for instance in the period between clinical trials demonstrating their efficacy and NICE guidance recommending their use?

Question 5: What do you think should happen to patients receiving treatment with a medicine under this scheme if the medicine subsequently fails to be granted a marketing authorisation?

We believe that people in this situation should be allowed to continue receiving the medicine if they wish to. This need not represent a major cost for the manufacturer (or whoever ends up paying for the drug): some people may wish to discontinue the treatment, while others may live only a short time after the decision, if the scope of the scheme is indeed focused on people with life threatening or seriously debilitating conditions.

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October 2012