Marie Curie Research Grants Scheme

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A prospective observation of secretion problems in motor neuron disease (ProSec)

Duration of project: 30 months

Start date: 1st May 2017

Budget: £249,456*
*Jointly funded by Marie Curie and the Motor Neurone Disease Association (50:50)

Abstract

Background

Motor Neuron Disease (MND) is a progressive neurodegenerative disease affecting the motor nerves supplying the limbs, trunk, bulbar region and respiratory muscles. It has been estimated that 50% of MND patients suffer from saliva problems and a recent survey of clinicians estimated that in 42% of patients, these problems are poorly controlled. Consequences include breakdown of the skin around the mouth, speech disturbance, disruption of sleep, coughing and a higher risk of aspiration. Additionally, drooling of saliva can lead to psychosocial symptoms including distress, embarrassment and social withdrawal. Management is usually determined by clinician experience and includes the use of anticholinergics, botulinum toxin, radiotherapy, surgery and non-pharmacological methods. However, the studies evaluating these therapies are to an extent limited by lack of blinding, few participants and the use of outcome measures not designed for patients with MND. NICE has recently recommended that there is a need for a prospective cohort study in this area, to understand how specialists are using these treatments, in order to inform future comparative studies.

Work leading up to this application

We have established a collaboration within the MND UK clinics, in order to develop the evidence base for the management of secretion problems in MND. In a retrospective case note review study of 119 patients from 16 centres, we identified a considerable variation in practice, reflecting the lack of evidence-based guidance in the area. The use of 5 types of anticholinergics, salivary gland botulinum toxin injections, conservative management approaches and carbocisteine were reported. However, in the absence of a standardised follow up protocol or outcome measure being used to assess the patients, it was not possible to determine the extent of any symptomatic improvement or severity of any adverse effects. Recognising the need for a standardised method for assessing the outcomes of secretion treatments, we have developed the clinical saliva scale for MND (CSS-MND). The CSS-MND is an 8 item patient reported outcome measure. Response options differ from item to item but are all based on a four point scale and span from no saliva related problem to a severe saliva related problem. The overall score ranges from 0 – 32 with a higher score indicating greater problems with saliva. Two sub scales can be reported, one for thin saliva problems (items A-F), and one for thick secretion problems (items H-I). For patients using non-invasive ventilation the scale has an additional 2 items and an overall score ranging from 0-40. We have demonstrated that the CSS-MND score provides a strong indicator for

the impact of saliva problems on patients (good face validity), more so than simple measurements of saliva volume or extent of drooling.

Aims

1. Describe the incidence of thin and thick secretion problems in the MND population
2. Describe the current management of thin and thick secretion problems in MND
3. Document the frequency of side effects of treatments for secretion problems
4. Document the efficacy of treatments for secretion problems
5. Determine if the CSS-MND is a) responsive to meaningful change, b) reliable.

Methods

This is an original, prospective, multi-centre cohort study. Patients with MND (500) will be recruited from 17 MND centres. Inclusion criteria will be broad to maximise entry into the study. Patients will be recruited over a 21 month period and will have a minimum follow up of 3 months and a maximum follow up of 12 months. Data will be collected on MND history, secretion problems (including CSS-MND, modified Likert scale), drug history. Patients who are stable will complete the CSS-MND and a global rating of change questionnaire 2-10 days following their baseline visit, to assess the test-retest reliability of the CSS-MND.

Expected outputs

The findings will provide a comprehensive description of the burden of saliva problems for patients living with MND and how they are currently managed. The analysis will provide an indication of the tolerability and side effects of the various treatment options in the MND population and will give some preliminary indication of efficacy. This will be an improvement on the current situation. Going forward the study will enable further refinement of the CSS-MND and confirm its validity and reliability. Together the findings will enable hypothesis generation and provide the tools for subsequent randomised trials.