Botulinum toxin for people with dystonia treated by an outreach nurse practitioner: a comparative study between a home and a clinic treatment service

Whitaker J, Butler A, Semlyen J K, Barnes M P

Record Status
This is a critical abstract of an economic evaluation that meets the criteria for inclusion on NHS EED. Each abstract contains a brief summary of the methods, the results and conclusions followed by a detailed critical assessment on the reliability of the study and the conclusions drawn.

Health technology
The use of a trained outreach nurse practitioner to give injections of botulinum toxin to patients with dystonia.

Type of intervention
Other: treatment service.

Economic study type
Cost-effectiveness analysis.

Study population
The study population was selected using two main inclusion criteria. First, there was a definite clinical diagnosis of spasmodic torticollis, blepharospasm, hemifacial spasm, or other segmental dystonia, hemidystonia, or generalised dystonia. Second, dystonia had been treated with botulinum toxin injections on at least two preceding occasions, and there was a clinical need for such injections to continue.

Patients who were unable to travel on a regular basis to the outpatient clinic were excluded, as were women in pregnancy or of childbearing potential. Patients were also excluded if they had psychiatric or other psychologic problems affecting compliance with the study protocol, if they had a known allergy to botulinum toxin, or had had side effects or another reaction to botulinum toxin. Patients with complex or variable dystonic movement disorder, requiring variations in the muscle injected or significant variations in other treatments on a visit-by-visit basis, were also excluded.

Setting
The setting was a hospital (outpatient). The economic study was carried out at the Academic Unit of Neurological Rehabilitation, Hunter Moor Regional Neurorehabilitation Centre, Newcastle upon Tyne, UK.

Dates to which data relate
No dates were reported.

Source of effectiveness data
The effectiveness evidence was derived from a single study.

Link between effectiveness and cost data
The costing was undertaken prospectively on the same patient sample as that used in the effectiveness analysis.
Study sample
No power calculations to determine sample size were performed. The patients were identified from the database at the study hospital. Of the 99 eligible patients, 10 refused to participate in the study. Only 2 patients declined to participate because they preferred to continue being seen in the clinic rather than receiving home-based treatment. The final sample comprised 89 patients enrolled during a 6-month period. There were 45 patients in the home group and 44 patients in the clinic group. In the home group, the mean time since onset was 14 years (range: 3 - 59) and 14 were men. In the clinic group, the mean time since onset was 14 years (range: 3 - 40 years) and 11 were men.

Study design
This was an open, randomised controlled trial, which was conducted in a single centre (the Academic Unit of Neurological Rehabilitation, Hunter Moor Regional Neurorehabilitation Centre). Randomisation was carried out using a random number table. The patients were followed for up to 18 months. No loss to follow-up was reported. To reduce any bias in assessment, the nurse was followed by a second research associate, who conducted a separate study and administered a User Satisfaction Questionnaire.

Analysis of effectiveness
All patients included in the study were accounted for in the analysis, implying that it was performed on an intention to treat basis. The health outcomes assessed were:

the number of total visits and total treatments,

mean relief,

mean attendance interval,

secondary toxin failures,

remissions,

missed appointments,

acute hospital admissions,

patients referred back to clinic,

the total number of reported incidents of side effects (diplopia, ptosis, dysphagia, pain at injection site, flu-like illness, and significant muscle weakness), and

the number of external referrals.

Finally, the patients were given the User Satisfaction Questionnaire. This asked whether there was any improvement in treatment and service, and which service (home or clinic) was preferred. The study groups were reported to have been comparable at baseline in terms of their demographics and medical parameters.

Effectiveness results
The numbers of total visits was 243 in the home group and 210 in the clinic group. The number of total treatments was 227 in the home group and 192 in the clinic group.

The mean relief changed from 8.1 weeks (range: 4 - 14) to 7.6 weeks (range: 4 - 14) in the home group, and from 8.2 weeks (range: 4 - 14) to 7.7 weeks (range: 4 - 14) in the clinic group.

The mean attendance interval changed from 10.6 weeks (range: 6 - 16) to 9.6 weeks (range: 6 - 14) in the home group, and from 11.5 weeks (range: 6 - 16) to 12.0 weeks (range: 6 - 16) in the clinic group.
There were 3 secondary toxin failures in the home group and 5 in the clinic group.

In the home group, there were 0 remissions, 5 missed appointments, 1 acute hospital admission, and 1 patient referred back to the clinic. In the clinic group, there was 1 remission, 6 missed appointments, 0 acute hospital admissions, and 0 patients referred back to the clinic.

No statistically significant difference was found in these outcomes.

The number of reported occurrences of side effects was generally similar among the study groups. The exception was the number of dysphagia episodes, which were 7 in the home group and 24 in the clinic group, (p<0.018).

There were 24 incidences of side effects in the home group and 40 in the clinic group, (p<0.041).

External referrals were more numerous in the home group, but the difference did not achieve statistical significance.

From the results of the questionnaire, 33 patients in the home group and 13 patients in the clinic group reported improvements in treatment, while 12 patients in the home group and 31 patients in the clinic group reported that the treatment was the same or worse, (p<0.001).

For 36 patients in the home group and 15 patients in the clinic group there were improvements in the service, while for 9 patients in the home group and 29 patients in the clinic group, the service was the same or worse, (p<0.001).

Among patients in the home group, 82% preferred the home service, nobody preferred the clinic, and 18% did not express any preference.

Clinical conclusions
The effectiveness analysis showed that the trained outreach nurse practitioner was as safe and effective as the clinic-based service for injections of botulinum toxin. However, the patients far preferred the service carried out at home by the trained outreach nurse practitioner.

Measure of benefits used in the economic analysis
The health outcomes were left disaggregated and no summary benefit measure was used in the economic analysis. A cost-consequences analysis was therefore carried out.

Direct costs
Discounting was not carried out since the costs were incurred over less than two years. The unit costs and the quantities of resources were not reported separately. The cost items included in the analysis were for the nurse’s travel and time, medical time, and the drug. The cost/resource boundary for direct costs was that of the National Health Service (NHS). The costs and the quantities were both estimated using actual data, derived from NHS rates. No price year was reported.

Statistical analysis of costs
No statistical analyses of the total costs were carried out.

Indirect Costs
The costs were not discounted due to the time horizon of the study (18 months). The unit costs and the quantities of resources were not reported separately. The costs of the clients’ time lost from work and travel were included in the analysis, reflecting the overall perspective of society. The cost and the quantity data were derived from gross salaries and standard NHS reimbursement rates for mileage incurred if using a car, or direct reimbursement of public transport rates. No price year was reported.
Currency
UK pounds sterling (£). These were converted to US dollars ($). The conversion rate was 1 = $1.58.

Sensitivity analysis
No sensitivity analyses were carried out.

Estimated benefits used in the economic analysis
See the 'Effectiveness Results' section.

Cost results
The direct NHS cost per visit was $32.40 (20.53) in the home group and $25.40 (16.08) in the clinic group.

The total costs per visit were $36.90 (23.36) in the home group and $79.00 (50.01) in the clinic group when the costs of the toxin were excluded. These rose to $306.20 (193.80) in the home group and $323.70 (204.88) in the clinic group when the costs of the toxin were included.

Synthesis of costs and benefits
Not relevant.

Authors' conclusions
The outreach nurse practitioner service was as effective and safe as the standard clinic-based service, and the patients preferred it. Although the NHS costs were slightly higher in the nurse practitioner group, the overall costs for society were lower than in the clinic-based service.

CRD COMMENTARY - Selection of comparators
The rationale for the choice of the comparator was clear. Clinic-based treatment was selected as it represented the standard service for the treatment of patients with dystonia. You should assess whether it represents a widely used service in your own setting.

Validity of estimate of measure of effectiveness
The effectiveness analysis was carried out using a prospective, randomised design, thus enhancing the internal validity of the study. The study sample appears to have been representative of the study population. The patients were followed for up to 18 months and no loss to follow-up was reported. The study groups were shown to be comparable at baseline. However, power calculations were not performed, and the dates during which the data were collected were not reported.

Validity of estimate of measure of benefit
No summary benefit measure was used and, hence, a cost-consequences analysis was carried out. Although the patients’ preferences were assessed, it would have been useful had the authors adopted a summary benefit measure, as numerous health outcome measures were reported.

Validity of estimate of costs
The economic analysis was carried out from a societal perspective, and all the relevant categories of costs were included. The direct costs were assessed on the basis of NHS data. The cost estimates were reported in US dollars, after appropriate conversions from UK pounds sterling. However, the costs and the quantities were treated deterministically and no statistical analyses were carried out. No price year was reported, thus making any reflation exercise to other settings difficult. The unit costs and the quantities were not reported separately, thus reducing transparency and
Other issues
The authors did not compare their findings with those from other studies. The issue of the generalisability of the study results to other settings was not addressed and sensitivity analyses were not carried out. Thus, the external validity of the analysis was somewhat low. Patients with dystonia were selected for the study and this was reflected in the authors’ conclusions. The effectiveness results of the study were reported satisfactorily, but the costing could have been reported in more detail.

Implications of the study
The authors recommend the development of outreach nurse practitioners, as suggested from the evidence collected in the study.

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