Aquatic therapy for children with Duchenne muscular dystrophy: a pilot feasibility randomised controlled trial and mixed-methods process evaluation

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Scientific summary

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Scientific summary

Background

Duchenne muscular dystrophy (DMD) is a rare disease that mainly affects boys. Among other effects, it causes progressive skeletal muscle weakness, which results in the loss of motor abilities such as walking. For many years, treatment has included daily physiotherapy. There is no randomised controlled trial (RCT) evaluating whether or not adding aquatic therapy (AT) to land-based exercises helps to keep muscles strong and boys with DMD independent.

Objectives

The primary objective was to determine the feasibility of recruitment to a full-scale trial, through recruiting 40 participants in 6 months from four centres, with two centres in reserve. The secondary objectives were to assess how, and how well, the intervention and trial procedures work.

Design

Development and formative evaluation of a complex intervention. Two-arm, parallel-group, external pilot randomised trial, with a 1 : 1 allocation ratio, using web-based randomisation and with only the principal investigator and analysts blind to allocation until after the final analysis. Intervention optimisation, qualitative research and health economic substudies.

Setting

Six paediatric neuromuscular units in the UK.

Participants

Between 24 October 2014 and 30 June 2015, 348 boys were screened for eligibility. The eligibility criteria included having genetically confirmed DMD, a North Star Ambulatory Assessment (NSAA) score of 8–34, being aged 7–16 years, being established on glucocorticoid corticosteroids and able to complete a 10-m walk test with no walking aids or assistance. Thirteen boys consented and 12 were randomised (30% of the recruitment target, n = 40) to AT plus land-based therapy (LBT) (n = 8) or LBT alone (n = 4).

Interventions

First, manualised AT, delivered by an AT-trained physiotherapist, in a 30-minute session, twice per week, in a NHS pool heated to a temperature of 34–36 °C plus LBT. A manual provided a menu of aquatic exercises using the properties of water (buoyancy, turbulence) including (1) active assisted and/or passive stretching that targets key muscle groups, (2) simulated or real functional activities and (3) submaximal exercise.

Second, manualised LBT, prescribed by a specialist physiotherapist at baseline, tailored to the capability and needs of the participant, with best practice advocating a regular stretching regime delivered by parents (4–6 days per week, physiotherapists advised participants not to complete LBT and AT on the same day).
targeting key muscle groups, plus a directed programme of exercises and advice on regular activity designed to prevent disuse atrophy.

Main outcome measures

Feasibility outcomes

1. Feasibility of recruitment to the main trial [recruitment of 40 participants in 6 months from four centres, with two in reserve (primary outcome)].
2. Decision on primary end point for main trial.
3. Feasibility of recruiting participating centres.
4. Number/characteristics of eligible patients approached, consenting, randomised and followed up, with reasons for refusal of consent and attrition.
5. Data completeness.
6. Independent assessment of whether AT or LBT was optimised.
7. Participant, parent and physiotherapist views on the acceptability of research and intervention procedures.

Clinical outcomes

For all participants:

1. NSAA
2. 6-minute walk distance (6MWD)
3. forced vital capacity
4. Activity Limitations Measure
5. Child Health Utility 9D Index – health state utility
6. the Care-related Quality of Life (carer burden) questionnaire
7. health and social care resource use questionnaire.

For participants allocated to AT only, at the end of each session:

1. pain (visual analogue scale)
2. Children’s OMNI Scale of Perceived Exertion.

Barriers to implementation of the trial and intervention were assessed using e-mail communication and Trial Management Group meeting minutes. Views on the acceptability of the AT and research protocols were obtained through semistructured interviews with participants, parents (n = 8 children) and health professionals (n = 8). Interviews were audio-recorded and transcribed verbatim with transcripts coded in NVivo version 11 (QSR International, Warrington, UK) and analysed using Framework analysis.

An independent rater reviewed baseline data and patient records (medical, social and school history) to determine the extent to which treatment was optimised, using attendance logs, the therapist-completed AT exercise log and the parent-completed LBT log.

A cost analysis was performed. Quantitative and qualitative data were mixed using a triangulation exercise.

Results

Over 6 months, 348 boys were screened, most of whom lived too far from centres or were enrolled in other trials; 13 consented and 12 were randomised (30% of target) to AT (n = 8) or control (n = 4). Two participants withdrew from, and one was lost to follow-up in, the control arm. As a result, the
were £80 based on attendance; societal costs ranged from £2541 to £3775. The estimated direct NHS costs of LBT provision at a tertiary centre ranged from £1970 to £2734 over 6 months, that were too extensive and insufficiently focused.

Physiotherapists and parents valued AT and believed that it should be delivered in community settings. The independent rater considered AT optimised for three out of eight boys, with other boys given programmes in patient-convenient, settings, where resources permitted.

Physiotherapists and parents valued AT and believed that it should be delivered in community settings. The independent rater considered AT optimised for three out of eight boys, with other boys given programmes that were too extensive and insufficiently focused.

Estimated direct NHS costs of AT provision at a tertiary centre ranged from £1970 to £2734 over 6 months, based on attendance; societal costs ranged from £2541 to £3775. The estimated direct NHS costs of LBT were £80–320, depending on the frequency with which the physiotherapist saw the boys; societal costs...
ranged from £732 to £1094. This could compare unfavourably with other specialist paediatric services, but delivery in the community could reduce the costs substantially.

Conclusions

Neither a full-scale RCT, designed on frequentist lines and recruiting in the UK alone nor a twice-weekly open-ended AT course delivered at tertiary centres is likely to be feasible. Many of the barriers that we encountered in the delivery of AT may not be encountered to the same extent if the intervention was delivered more locally to the service user and in community settings. Further intervention development research is needed to identify how community-based pools can be accessed and how DMD families can link with each other as well as community physiotherapists who can tailor AT programmes guided by highly specialised physiotherapists from tertiary centres. Bayesian RCTs may be able to reduce sample sizes such that UK-based recruitment is feasible over 2–3 years. Future studies should use the routinely collected NSAA score as a primary outcome.

Trial registration

This trial is registered as ISRCTN41002956.

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