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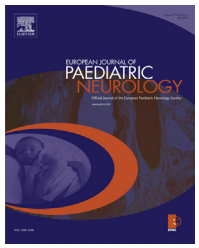

Original article

Long-term follow-up on continuous intrathecal Baclofen therapy in non-ambulant children with intractable spastic Cerebral Palsy

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ABSTRACT

Background: Little is known about the long-term effects of Continuous intrathecal Baclofen (CITB) therapy in non-ambulant children with intractable spastic Cerebral Palsy (CP).
Aim: To determine whether short-term beneficial effects of CITB therapy are present at the long-term, and whether caregivers would choose CITB therapy for their child again considering the advantages and disadvantages encountered over the years.
Methods: Long-term follow-up data were obtained of the children whom had previously participated in a RCT on CITB by the Dutch Study Group on Spasticity. Quality of life (QoL) was assessed by the Child Health Questionnaire (CHQ), current satisfaction with CITB was measured by use of a Visual Analogue Scale regarding previously set treatment goals, functioning in daily living was determined by a questionnaire concerning functioning of the child, and possible detrimental effects of CITB therapy encountered over the years were noted. All data were acquired via interview of the caregivers.
Results: All 17 children of the former trial participated in this study. Previously identified significant positive effects on pain (CHQ 46.8 vs. 74.38, $p = 0.002$; VAS 2.4 vs. 8.01, $p = 0.02$), ease of care (VAS 2.0 vs. 7.26, $p = 0.00$), and mental health (CHQ 67.2 vs. 75.94, $p = 0.010$) were still present at the end of the trial. Novel significant positive effects were noted at six to nine years follow-up, i.e. significantly improved scores on the Parent Impact – Emotional subscale (CHQ 66.0 vs. 78.2, $p = 0.008$), Parent Impact – Time subscale (CHQ 68.9 vs. 91.72, $p = 0.002$), and the Physical Summary (CHQ 17.6 vs. 27.4, $p = 0.019$)

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compared to baseline. Ninety-four percent of the caregivers would choose CITB treatment again for their child again.

Conclusion: The beneficial effects of CITB are present at the long term and caregiver satisfaction is high.

1. Background

Continuous Intrathecal Baclofen (CITB) therapy, i.e. the direct continuous administration of Baclofen into the intrathecal space, is an effective treatment modality of intractable spasticity in non-ambulant children with Cerebral Palsy (CP).[1,2] In a study with a prospective, randomized with open blind endpoint evaluation (PROBE) design we (on behalf of the Dutch Study Group on Spasticity) showed that CITB relieved pain, facilitated ease of care, and improved mental health in 17 children during a mean follow-up of 18.4 months.[3–5] Our findings were supported by others.[6,7]

Despite these positive effects, long-term follow-up data on treatment effects are still currently unavailable. Nevertheless, these data are needed as this treatment modality is most likely to be installed for decades.[8] It has been argued to use a single-participant experimental design that uses the individual as his own comparison as an option for future research.[9]

Acting upon these remarks we report the long-term follow-up on the 17 children of the trial by the Dutch Study Group on Spasticity.[3–5] The aim of this study was to determine whether beneficial effects of CITB therapy are still present six to nine years after commencement of treatment, and whether caregivers would still choose this treatment modality for their child today considering the advantages and disadvantages of CITB therapy encountered over the years.

2. Methods

2.1. Design

Prospective follow-up cohort study on the results of CITB in 17 children with intractable spastic CP. The caregivers of the children were informed about the study and all gave informed consent. This study was approved by the Medical Ethics Committee of the Maastricht University Medical Centre (MUMC) according to Dutch governmental guidelines.

2.2. Participants

All 17 children of the trail by the Dutch Study Group on Spasticity participated.

In summary, at the beginning of this study, thirty-eight children (23 boys and 15 girls) were considered possible candidates by The Dutch Study Group on Spasticity. Children were referred to our clinic because of pain and problems of care. Seventeen children met the inclusion criteria. At time of inclusion the group consisted of nine girls and eight boys; mean age 13 years and 2 months (SD 2 years and 9 months, 9–16 years). All were tetraplegic, except for three who were diplegic. All were spastic and five were spastic as well as dyskinetic. All children were Gross Motor Function Classification System (GMFCS) level 5, except for two who were level 4, and one who was level 3. Weight varied between 17 and 84 kg. See references three to five for more detailed information.[3–5]

2.3. Outcome measures

Primary outcome measures were quality of life (QoL) and satisfaction on the initially and individually defined treatment goals of these children. These individually defined treatment goals were set at the beginning of the trial.[3–5] QoL was measured by means of the Child Health Questionnaire (CHQ), whereas a Visual Analogue Scale (VAS) was chosen to measure caregivers' satisfaction for the initially defined treatment goals. Both methods are sensitive in detecting change.[3–5]

The CHQ is a generic QoL instrument which measures 14 unique physical and psychosocial concepts.[10] In this present study the 50-item, parent-form was used (CHQ-PF50). All domains are scored on a 0–100 scale with higher scores reflecting better QoL. Physical and psychosocial summary scores can be derived from the separate CHQ profile scores, which have a mean of 50 and an SD of 10 in the general population.

To measure satisfaction on defined treatment goals a VAS was used. This is a straight 10-cm horizontal line with the anchor points "very satisfied" (score 10) and "very dissatisfied" (score 0). This is opposite to how the VAS is commonly used, but in line with the previous studies of the Dutch Study Group on Spasticity.[3–5] Although, the caregivers were reminded of the three treatment goals defined before pump implantation, they were not informed about the previous scores. They scored their current satisfaction with regard to these goals on the VAS. The mean of these three VAS scores was used for statistical analysis (individual VAS). VAS scores for improvement of ease of care and relief of pain were analyzed separately as these were the most frequently formulated treatment goals.

Secondary outcome measures were level of functioning (worse or better) and the percentage of caregivers that would choose CITB therapy for their child today considering the advantages and disadvantages encountered over the years.

Caregivers had to fill out a questionnaire addressing daily functioning of their child on 40 domains. Domains are scored on a five-point Likert scale ("A lot worse" – "Slightly better" – "The same" – "Slightly better" – "A lot better"). This questionnaire was developed by Staal et al. who used it to identify areas of improvement after CITB therapy in 49 individuals.[11] It has also been used by Krach et al. in order to evaluate satisfaction regarding CITB therapy in 100 patients/caregivers.[12] This questionnaire was carefully translated into Dutch.

Furthermore, caregivers had to answer questions with regard to pain caused by the CITB pump, side-effects at present time,