Child Health Questionnaire (CHQ) Clinical Trials References

Abstract: OBJECTIVE: The aim of this study was to study treatment response to atomoxetine in a large, multicenter study of non-North American patients with ADHD. METHODS: A total of 604 children and adolescents with ADHD were enrolled in a 10-week open-label trial with atomoxetine prior to randomization to a double-blind relapse prevention phase at 33 sites in the United Kingdom, continental Europe, Israel, South Africa, and Australia. All patients had ADHD symptom severity at least 1.5 standard deviations above United States age and gender norms for their diagnostic subtype as measured by the investigator-scored ADHD Rating Scale (ADHD RS). Outcomes were assessed by analysis of change in the ADHD RS; functional and psychosocial outcomes were assessed using the Child Health Questionnaire (CHQ). RESULTS: At endpoint, ADHD RS total scores decreased by an average of 56.7%, and 69% of patients were rated as having no or minimal symptoms. Significant improvement was observed in psychosocial and functional outcomes. Discontinuations attributed to adverse events were < 4%. CONCLUSION: These open-label data, gathered in an international setting, add to our knowledge of the value of atomoxetine in treating ADHD symptoms, as well as its safety and tolerability.

Abstract: OBJECTIVE: To assess the safety, tolerability, and efficacy of GW320659, a chemically novel inhibitor of norepinephrine and dopamine reuptake, in pediatric attention-deficit/hyperactivity disorder (ADHD). METHOD: This was a multicenter, open-label, dose-titration study of seven daily dose levels of GW320659: 1.25, 2.5, 5, 7.5, 10, 12.5, and 15 mg. Treatment began with the lowest dose of GW320659 and increased weekly until subjects (mean age 9.1 years) achieved a maximum acceptable dose. Subjects remained at their maximum acceptable dose for a 4-week treatment period. The key efficacy end-point was clinical response (Clinical Global Impressions of Improvement score of 1 or 2 and an improvement of 5 or more points on at least one of the Conners Parent or Teacher Rating Scales Tscore). Other end-points included assessments of safety and of quality of life using the Child Health Questionnaire Parent Form 28 (CHQ-PF28). RESULTS: Fifty-one subjects entered the titration phase and 46 subjects completed the study. During the treatment phase, these 46 subjects received a mean dose of 14.2 mg/day and the maximum exposure to GW320659 was 11 weeks. At the end of the treatment period, 76% of subjects showed improvement with GW320659 and there were significant improvements in 7 of the 12 subscales of the CHQ-PF28 compared with baseline (p < .05). Adverse events were generally mild; only five subjects required downward titration because of adverse events (three psychiatric, one neurological and urological, one cardiovascular), and no subject withdrew because of adverse events. CONCLUSIONS: GW320659 may have clinically relevant efficacy in pediatric ADHD and was well tolerated in this short-term initial study in children.

Abstract: BACKGROUND: The current study was conducted to assess the safety and tolerability of a transdermal fentanyl delivery system for the relief of chronic pain in a pediatric population,
and also to validate titration recommendations and conversion to transdermal fentanyl from oral opioid therapy. METHODS: This 15-day (with 3-month extension), single-arm, open-label trial was conducted at 66 sites in 10 countries. A total of 199 pediatric patients (ages 2-16 years) with both malignant and nonmalignant conditions who were receiving oral or parenteral opioids for moderate to severe chronic pain were enrolled. Transdermal fentanyl doses were titrated upward according to the rescue medication consumed during the previous application period. Degree of pain was assessed by patients and parents/guardians using visual and numeric scales. Level of play and quality of life were assessed using the Play Performance Scale (PPS) and the Child Health Questionnaire (CHQ). Adverse events were monitored on Days 1-15. Hypoventilation and sedation were monitored every 4 hours during the first 72 hours of the study. RESULTS: A total of 173 patients completed the primary treatment period and 130 entered the extension phase. The average daily pain intensity scores were reported to have decreased by Day 16 and improvements in the mean PPS scores were observed to the end of the extension period. The CHQ scores demonstrated improvements in 11 of 12 domains after Month 1 of the extension period. CONCLUSIONS: Transdermal fentanyl was found to be a safe and well tolerated alternative to oral opioid treatment for children ages 2-16 years who were previously exposed to opioid therapy.


Abstract: Purpose As the number of pediatric cancer survivors increases, so does the number of survivors previously exposed to anthracyclines as part of their cancer therapy. Because screening is costly, some have suggested that health-related quality of life (HRQL) measures might be useful in focusing screening tests on those patients with cases most likely to display positive findings. This study reports on the predictive ability of HRQL measures to detect patients with abnormalities on serial cardiac testing. Methods Using 127 patients from the ACE-Inhibitor after Anthracycline (AAA) Trial, this study compared serial measures of the Short Form-36 (SF-36; for ages > 13 years) and Child Health Questionnaire-Child Form 87 (CHQ-CF87; for ages less than or equal to 13 years) to serial cardiac performance tests including echocardiographic shortening fraction, left ventricular end systolic wall stress (LVESWS), LVESWS-index, and maximal cardiac index (MCI; a measure of cardiac output at peak exercise). Results Generally, there was no clinically or statistically significant correlation between any HRQL measure and any cardiac function measure except between MCI and vitality and physical functioning. For each of these measures, the correlation between MCI was statistically significant (P < .006), but each HRQL subscale could explain no more than 7% of the variation in MCI. HRQL measures were not predictive of any other cardiac function measure. Conclusion HRQL measures should not be used in isolation as a screen for cardiac function abnormalities in patients exposed to anthracyclines who already have a mild degree of ventricular dysfunction. Patient history appears to be no substitute for cardiac-testing in this cohort.


Abstract: Clinical research on attention-deficit hyperactivity disorder (ADHD) has begun to integrate measures of health-related quality of life (HRQL) as part of the overall assessment of treatment outcomes. This study examines the association between HRQL and measures of clinical symptoms of ADHD. Data were gathered from 297 children and adolescents in an 8-week, randomized, double-blind, placebo-controlled, clinical trial of atomoxetine treatment for ADHD. HRQL was assessed with the Child Health Questionnaire 50-item Parent Form. ADHD symptoms were assessed with the ADHD Rating Scale-IV; Parent Version and Clinical Global Impressions-ADHD-Severity. Associations between HRQL and clinical symptoms were assessed with
correlations, analyses of variance with post hoc comparisons, and t tests. The Child Health Questionnaire 50-item Parent Form scales assessing psychosocial domains of HRQL were significantly negatively correlated with clinical measures. Improvement in clinical symptoms was associated with corresponding improvement in psychosocial aspects of HRQL. The findings suggest that HRQL instruments can add important information to efficacy measures in clinical trials of ADHD treatment.


Abstract: OBJECTIVE: Atomoxetine is an investigational, nonstimulant pharmacotherapy being studied as potential treatment for attention-deficit/hyperactivity disorder (ADHD). It is thought to act via blockade of the presynaptic norepinephrine transporter in the brain. We assessed the efficacy of 3 doses of atomoxetine compared with placebo in children and adolescents with ADHD. METHODS: A total of 297 children and adolescents who were 8 to 18 years of age and had ADHD as defined by the Diagnostic and Statistical Manual of Mental Disorders, 4th edition, were randomized to placebo or atomoxetine dosed on a weight-adjusted basis at 0.5 mg/kg/day, 1.2 mg/kg/day, or 1.8 mg/kg/day for an 8-week period. ADHD symptoms, affective symptoms, and social and family functioning were assessed using parent and investigator rating scales.

RESULTS: Approximately 71% of children enrolled were male, approximately 67% met criteria for mixed subtype (both inattentive and hyperactive/impulsive symptoms), and the only common psychiatric comorbidity was oppositional defiant disorder (approximately 38% of the sample). At baseline, symptom severity was rated as moderate to severe for most children. At endpoint, atomoxetine 1.2 mg/kg/day and 1.8 mg/kg/day were consistently associated with superior outcomes in ADHD symptoms compared with placebo and were not different from each other. The dose of 0.5 mg/kg/day was associated with intermediate efficacy between placebo and the 2 higher doses, suggesting a graded dose-response. Social and family functioning also were improved in the atomoxetine groups compared with placebo with statistically significant improvements in measures of children's ability to meet psychosocial role expectations and parental impact. Discontinuations as a result of adverse events were <5% for all groups.

CONCLUSION: Among children and adolescents aged 8 to 18, atomoxetine was superior to placebo in reducing ADHD symptoms and in improving social and family functioning symptoms. Atomoxetine was associated with a graded dose-response, and 1.2 mg/kg/day seems to be as effective as 1.8 mg/kg/day and is likely to be the appropriate initial target dose for most patients. Treatment with atomoxetine was safe and well tolerated.


Abstract: OBJECTIVE: To compare the effects of 2 nebulizable controller asthma medications on caregiver and pediatric quality of life. METHODS: In this 52-week, randomized trial, children aged 2 to 6 years with mild to moderate persistent asthma received budesonide inhalation suspension 0.5 mg (total daily dose) once or twice daily (n = 168) or cromolyn sodium nebulizer solution 20 mg 4 times daily (n = 167) for 8 weeks, with dosage adjustment thereafter at the investigators' discretion. The Pediatric Asthma Caregiver's Quality of Life Questionnaire (PACQLQ), Compliance/Caregiver Satisfaction Questionnaire (CCSQ), Modified Child Health Questionnaire-Parent Form 50 (CHQ-PF50), and Functional Status-II(R) (FS-II(R)) Questionnaire were administered at baseline and weeks 8, 28, and 52. Global assessments of ease of asthma management and child health status were obtained from caregivers and physicians at the end of the study. RESULTS: Improvements from baseline in domain-specific (activities and emotional function) and total PACQLQ scores were greater at each time point (weeks 8, 28, and 52) for caregivers of patients treated with budesonide compared with caregivers of patients receiving cromolyn sodium. Only the budesonide group met the criterion for a clinically important improvement (>or=0.5 unit change) in all PACQLQ domains by week 8, which was maintained at weeks 28 and 52. Moreover, improvements surpassed the criterion for moderate clinical
importance (1.0 unit change) in all PACQLQ domains for the budesonide group, but this level of improvement was only achieved in the activities domain (at week 28) for the cromolyn sodium group. Based on the CCSQ, budesonide resulted in greater caregiver satisfaction, treatment convenience, ease of use, and compliance compared with cromolyn sodium. Thus, 90.7% of caregivers in the budesonide group were "completely or very satisfied" compared with 53.4% in the cromolyn sodium group. Over half (54.6%) of caregivers in the budesonide group rated budesonide "highly or very convenient" compared with 23% for cromolyn sodium; 77% rated budesonide "extremely or very easy" to use compared with 47% for cromolyn. Adherence with daily medication regimens was reported for 76% of children in the budesonide group compared with 57% in the cromolyn sodium group. Child health status, as indicated by mean FS-II(R) scores, showed improvements from baseline in both groups at weeks 8, 28, and 52. There was a trend for these improvements to be superior in the budesonide group. Additionally, budesonide was superior to cromolyn sodium in caregiver and physician global assessments. At the end of the study, 76% of caregivers of children receiving budesonide reported asthma management to be "a great deal easier" compared with the start of the study, and 74% rated the overall health status of their child as "much better now than 1 year ago." In contrast, only 29% and 37% of caregivers whose children received cromolyn sodium provided these respective ratings.

CONCLUSIONS: Budesonide inhalation suspension improved the quality of life for caregivers of children with asthma. Caregivers of children treated with budesonide had significantly fewer limitations in daily activities and emotional functioning compared with caregivers of children treated with cromolyn sodium nebulizer solution. The improvements in caregiver quality of life occurred earlier with budesonide compared with cromolyn sodium. Only caregivers in the budesonide group had a clinically important mean change from baseline in all PACQLQ domains by week 8. These benefits were maintained at week 52. Children treated with budesonide inhalation suspension and cromolyn sodium experienced improvements in health status, assessed using the FS-II(R). The greatest differences between treatments were seen in the disease-specific portion of the FS-II(R), which relates impairments in functional status to the child's illness. Caregiver and physician global assessment indicated significantly better overall child health after 1 year of treatment with budesonide, supporting an improvement in health status. Clinical trials in children 4 to 16 years of age with asthma have demonstrated greater effectiveness of inhaled corticosteroids versus cromolyn sodium on several clinical measures of efficacy. Measures of asthma control in this study, reported in detail elsewhere [Leflein et al. Pediatrics 2002;109:866-872], also have shown greater improvements with budesonide therapy. Treatment with budesonide inhalation suspension resulted in a significantly lower mean rate of asthma exacerbations, significantly longer times to first asthma exacerbation, significantly longer times to first additional use of chronic asthma therapy, and significant improvements in asthma symptom scores and breakthrough medication use compared with cromolyn sodium therapy. Additionally, children receiving budesonide inhalation suspension experienced more symptom-free days and episode-free days compared with children receiving cromolyn sodium. Safety profiles were similar between the 2 treatment groups. Budesonide inhalation suspension was associated with significantly greater caregiver satisfaction, convenience, ease of use, and compliance compared with cromolyn sodium nebulizer solution. This greater caregiver satisfaction and quality of life may be related to the greater asthma control achieved in children treated with budesonide therapy compared with cromolyn sodium. In addition, the convenience of once- or twice-daily dosing with budesonide inhalation suspension, compared with 3- or 4-times-daily dosing of cromolyn sodium, may decrease caregiver burden and enhance the willingness of caregivers to adhere to treatment regimens prescribed for their young children with asthma. This effect on caregiver adherence could further improve treatment effectiveness. This is the first clinical trial comparing the effects of a nebulized corticosteroid with that of an alternative nebulized therapy on quality of life in young children with asthma and their families. Compared with nebulized cromolyn sodium, budesonide inhalation suspension not only provides better overall child health status and asthma management, but greater caregiver quality of life and greater caregiver satisfaction, convenience, ease of use, and compliance

Newcorn JH, Spencer TJ, Biederman J et al. Atomoxetine treatment in children and adolescents with attention-deficit/hyperactivity disorder and comorbid oppositional
Abstract: OBJECTIVE: To examine (1) moderating effects of oppositional defiant disorder (ODD) on attention-deficit/hyperactivity disorder (ADHD) treatment response and (2) responses of ODD symptoms to atomoxetine. METHOD: Children and adolescents (ages 8-18) with ADHD were treated for approximately 8 weeks with placebo or atomoxetine (fixed dosing: 0.5, 1.2, or 1.8 mg/kg/day, b.i.d.) under randomized, double-blind conditions. Among patients with lifetime diagnostic information (n = 293), 39% were diagnosed with comorbid ODD and 61% were not. Treatment-group differences and differences between patients with and without comorbid ODD were examined post hoc for changes on the Attention-Deficit/Hyperactivity Disorder Rating Scale IV-Parent version, investigator-administered and -scored; Conners' Parent Rating Scale-Revised Short Form; Clinical Global Impressions Severity of ADHD Scale; and the parent-rated Child Health Questionnaire. RESULTS: Youths with ADHD and comorbid ODD showed statistically significant improvement in ADHD, ODD, and quality-of-life measures. Treatment response was similar in youths with and without ODD, except that the comorbid group showed improvement compared with placebo at 1.8 mg/kg/day but not 1.2 mg/kg/day. In contrast, youths without ODD showed improvement at 1.2 mg/kg/day and no incremental benefit at 1.8 mg/kg/day. CONCLUSIONS: Atomoxetine treatment improves ADHD and ODD symptoms in youths with ADHD and ODD, although the comorbid group may require higher doses

Abstract: Despite significant functional impairments associated with attention-deficit hyperactivity disorder (ADHD) and the growing appreciation of the importance of health-related quality of life (HRQL) assessment in children with chronic disorders, relatively few studies have examined the impact of ADHD treatment on HRQL. This investigation examines the effect of atomoxetine, a nonstimulant treatment for ADHD, on HRQL and identifies factors that are predictive of HRQL improvements. The Child Health Questionnaire (CHQ), which is a multidimensional HRQL measure, was collected during three randomized, double-blind, placebo-controlled clinical trials. Children who received atomoxetine had significantly greater improvement in psychosocial functioning compared to the placebo group. No significant differences between once-a-day and twice-a-day dosing were found. Treatment with atomoxetine, lower HRQL baseline score, no history of stimulant use, and absence of oppositional defiant disorder were all associated with improvements in psychosocial functioning. Findings demonstrate the positive impact of atomoxetine on HRQL in children with ADHD

Abstract: OBJECTIVES: We characterized a population-based cohort of school-aged children with severe hemophilia with respect to type of treatment, on-demand versus prophylaxis, and frequency of bleeding episodes in the year before enrollment. We also investigated the association between hemophilia-related morbidity, measured by number of bleeding episodes in the year before enrollment, and academic performance after adjustment for other factors known to have an effect on achievement. Finally, we explored the mechanisms for the association between bleeding episodes and academic achievement. STUDY DESIGN: This study was a multicenter investigation of boys 6 to 12 years old with severe factor VIII deficiency (clotting factor level <2%) receiving care in US hemophilia treatment centers. Children with a history of inhibitor, severe developmental disorder, significant psychiatric disorder, or insufficient fluency in English were excluded from the study. On-demand treatment was defined as administration of clotting factor on the occurrence of a bleeding episode. Prophylactic therapy was defined as a course of regular infusions for >2 months with a goal of preventing bleeding episodes. Academic achievement was measured by the Wechsler Individual Achievement Test. Quality of life was measured by the Child Health Questionnaire. Of particular interest was the Physical Summary (PhS) measure of the Child Health Questionnaire. The type of information captured by the PhS includes limitations in physical activity, limitations in the kind or amount of schoolwork or social activities the child engaged in, and presence of pain or discomfort. RESULTS: One hundred
thirty-one children were enrolled, a median center recruitment rate of 77%. The mean age of the participants was 9.6 years, and approximately half of the participants had completed less than the fourth grade at the time of enrollment. Sixty-two percent of the children were on prophylaxis at enrollment, and 9% had previously been on prophylaxis but were currently on on-demand therapy. Two groups were defined: ever treated with prophylaxis and never treated with prophylaxis. For those ever treated, treatment duration ranged from 2.7 months to 7.7 years, with one half of the children treated with prophylaxis for >40% of their lifetimes; 29% had always been on on-demand therapy. Children in both treatment groups were similar with respect to age, clotting factor level, parents' education, and IQ. The median number of bleeding episodes experienced in the year before enrollment for the cohort as a whole was 12. The median number of bleeding episodes in children on prophylaxis at enrollment was significantly lower than in children on on-demand therapy (6 vs 25.5). The mean achievement scores were within the average range of academic performance: reading, 100.4; mathematics, 101.6; language, 108.1; writing, 95.4; and total achievement, 102.5. When children were categorized as above or below the study group median by number of bleeding episodes, those who had a low number of bleeding episodes (< or =11) had better total achievement (104.4 vs 100.6) and mathematics (103.6 vs 99.6) than children in the higher bleeding episode category (> or =12) after adjusting for child's IQ and parents' education. Treatment with prophylaxis per se was not associated with better test scores, but children who had been treated on a regimen of long-term prophylaxis (>40% of lifetime) and reported < or =11 bleeding episodes in the year before enrollment had significantly higher scores in total achievement (104.9 vs 100.6), mathematics (105.2 vs 99.6), and reading (104.0 vs 98.6) than all other children reporting > or =12 bleeding episodes in the same time period. Increased school absenteeism and hemophilia-related limitations in physical functioning among children with greater frequency of bleeding episodes were proposed as the mechanisms for lower scores. The number of bleeding episodes was positively correlated with school absenteeism (Spearman correlation = 0.23), and children with more school absences had lower scores in mathematics, reading, and total achievement, even after adjusting for the child's IQ and parents' education. Children with fewer bleeding episodes also had better PhS scores than children in the high bleeding episode category (48.4 vs 41.3). The mean PhS for children in the low bleeding episode group (48.4) was similar to that of the general US population (50), but the mean PhS for children in the higher bleeding episode group was almost a full standard deviation lower than the mean for the general US population. PhS scores were positively related to reading and total achievement scores after adjusting for IQ and parents' education. Of interest and concern was a group of children who were reportedly being treated with prophylaxis during the year before enrollment (N = 18) but whose bleeding events were not optimally suppressed. These children were 3 times as likely (33.3% vs 11.1%) to be receiving < or =2 infusions per week as children on prophylaxis who reported < or =11 bleeding episodes during the same period. A review of the sites of bleeding reported for the 18 children revealed that 12 (66.6%) experienced > or =25% of their bleeding episodes in the same joint. CONCLUSIONS: Each child should have the opportunity to achieve his or her potential. Control of a chronic disorder must include this important goal as well as the more commonly identified medical outcomes. This study has identified an important association between the number of bleeding episodes experienced and academic achievement in a cohort of school-aged children with severe hemophilia. The data support the assertion that therapeutic care programs in this population must not be evaluated only in terms of financial cost to achieve adequate musculoskeletal outcomes. Also significant are the individual and societal benefits of increased academic accomplishments if adequate suppression of hemorrhagic events can be attained. The number of bleeding episodes experienced, regardless of treatment regimen, should be followed to optimize the child's academic outcome.


Abstract: OBJECTIVES: To test the hypotheses that virtual outreach would reduce offers of hospital follow-up appointments and reduce numbers of medical interventions and investigations, reduce numbers of contacts with the health care system, have a positive impact on patient
satisfaction and enablement, and lead to improvements in patient health status. To perform an economic evaluation of virtual outreach. DESIGN: A randomised controlled trial comparing joint teleconsultations between GPs, specialists and patients with standard outpatient referral. It was accompanied by an economic evaluation. SETTING: The trial was centred on the Royal Free Hampstead NHS Trust, London, and the Royal Shrewsbury Hospital Trust in Shropshire. The project teams recruited and trained a total of 134 GPs from 29 practices and 20 consultant specialists. PARTICIPANTS: In total, 3170 patients were referred, of whom 2094 consented to participate in the study and were eligible for inclusion. In all, 1051 patients were randomised to the virtual outreach group and 1043 to standard outpatient appointments. The patients were followed 6 months after their index consultation. INTERVENTIONS: Patients randomised to virtual outreach underwent a joint teleconsultation, in which they attended the general practice surgery where they and their GP consulted with a hospital specialist via a videolink between the hospital and the practice. MAIN OUTCOME MEASURES: Outcome measures included offers of follow-up outpatient appointments, numbers of tests, investigations, procedures, treatments and contacts with primary and secondary care, patient satisfaction (Ware Specific Visit Questionnaire), enablement (Patient Enablement Instrument) and quality of life (Short Form-12 and Child Health Questionnaire). An economic evaluation of the costs and consequences of the intervention was undertaken. Sensitivity analysis was used to test the robustness of the results. RESULTS: Patients in the virtual outreach group were more likely to be offered a follow-up appointment. Significant differences in effects were observed between the two sites and across different specialities. Virtual outreach increased the offers of follow-up appointments more in Shrewsbury than in London, and more in ENT and orthopaedics than in the other specialities. Fewer tests and investigations were ordered in the virtual outreach group, by an average of 0.79 per patient. In the 6-month period following the index consultation, there were no significant differences overall in number of contacts with general practice, outpatient visits, accident and emergency contacts, inpatient stays, day surgery and inpatient procedures or prescriptions between the randomised groups. Tests of interaction indicated that virtual outreach decreased the number of tests and investigations, particularly in patients referred to gastroenterology, and increased the number of outpatient visits, particularly in those referred to orthopaedics. Patient satisfaction was greater after a virtual outreach consultation than after a standard outpatient consultation, with no heterogeneity between specialities or sites. However, patient enablement after the index consultation, and the physical and psychological scores of the Short Form-12 for adults and the scores on the Child Health Questionnaire for children under 16, did not differ between the randomised groups at 6 months’ follow-up. NHS costs over 6 months were greater for the virtual outreach consultations than for conventional outpatients, pound 724 and pound 625 per patient, respectively. The index consultation accounted for this excess. Cost and time savings to patients were found. Estimated productivity losses were also less in the virtual outreach group. CONCLUSIONS: Virtual outreach consultations result in significantly higher levels of patient satisfaction than standard outpatient appointments and lead to substantial reductions in numbers of tests and investigations, but they are variably associated with increased rates of offer of follow-up according to speciality and site. Changes in costs and technological advances may improve the relative position of virtual consultations in future. The extent to which virtual outreach is implemented will probably be dependent on factors such as patient demand, costs, and the attitudes of staff working in general practice and hospital settings. Further research could involve long-term follow-up of patients in the virtual outreach trial to determine downstream outcomes and costs; further study into the effectiveness and costs of virtual outreach used for follow-up appointments, rather than first-time referrals; and whether the costs of virtual outreach could be substantially reduced without adversely affecting the quality of the consultation if nurses or other members of the primary care team were to undertake the hosting of the joint teleconsultations in place of the GP. Qualitative work into the attitudes of the patients, GPs and hospital specialists would also be valuable.


Abstract: OBJECTIVE: To describe the functional and family-centered assessment protocol and
outcomes of a phase II trial evaluating upper-limb function after botulinum toxin injections in children with cerebral palsy (CP). DESIGNS: Intervention study, case series, phase II trial, follow-up at 2 weeks and 3 and 6 months. SETTING: Specialist outpatient physical disabilities clinic within a public pediatric teaching hospital. PARTICIPANTS: Convenience sample of 16 children with CP (age range, 2-12y). INTERVENTIONS: Botulinum toxin type A (Botox) injections after electrical stimulation localization of appropriate muscle. MAIN OUTCOME MEASURES: The Canadian Occupational Performance Measure (COPM), Goal Attainment Scale (GAS), Melbourne Assessment of Unilateral Upper Limb Function, Child Health Questionnaire (CHQ), parent questionnaire, Modified Ashworth Scale (MAS), Tardieu scale, and active (AROM) and passive (PROM) range of motion. RESULTS: On the COPM, there was significant improvement at 3 months and 6 months. On the GAS, the T-scores were 42 and 47 at 3 and 6 months, respectively. On the Melbourne Assessment and CHQ, there was no significant change. The parent questionnaire indicated acceptability of injections and positive outcomes. On the MAS, there was a significant reduction in tone at 2 weeks, with a return to baseline by 6 months. On the Tardieu scale, there was a significant increase in angle of first catch at 2 weeks, but only the elbow maintained a significant difference at 3 and 6 months. No significant change was found for AROM or PROM. CONCLUSIONS: Sustained functional outcomes occurred after botulinum toxin injections despite increasing muscle tone after an initial reduction in tone. Randomized controlled trials are required.