HEALTH-RELATED QUALITY OF LIFE IMPROVEMENTS WITH DYSPORT IN CERVICAL DYSTONIA

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In a multicenter, double-blind trial, Dysport 500 units was compared to placebo in the treatment of cervical dystonia. The primary efficacy response was evaluated using the Toronto Western Spasmodic Torticollis Rating Scale (TWSTRS). Pain was evaluated with the Pain subscale of the TWSTRS and a self-reported pain analog scale (VAS). HRQoL was assessed using the SF-36 Health Survey (SF-36). OBJECTIVES: To evaluate improvements in health-related quality of life (HRQoL) with in patients with cervical dystonia enrolled in a randomized clinical trial with Dysport® (also known as abobotulinumtoxinA for Injection in the United States). METHODS: Eighty-eight patients were randomly assigned to receive one treatment with Dysport 500 units or placebo. Participants were assessed at baseline and weeks 2, 4, 8, 12, 16, and 20 after treatment. To evaluate HRQoL, changes from baseline to Week 8 on the 8 SF-36 domains, the TWSTRS Pain subscale, and the pain VAS were compared. RESULTS: TWSTRS total scores were significantly improved with Dysport at weeks 4, 8, and 12 (P < 0.013 when compared with placebo). Improvements from baseline to week 8 were seen for all 8 SF-36 domains in the Dysport group. The largest improvements occurred in the Role-Physical and Bodily Pain domains. The placebo group showed some decrease (worsening) in Physical Functioning and little to no change in other SF-36 domains. The differences in means between Dysport and placebo were statistically significant between the Dysport and placebo for 5 of the 8 domains (Physical Functioning, Role-Physical, Bodily Pain, General Health, and Role-Emotional (P ≤ 0.03 for all)). Improvement in the Bodily Pain domain was also supported by significant improvements in the TWSTRS Pain subscale at the pain VAS at week 4. CONCLUSIONS: The data suggest that Dysport is improved with Dysport, particularly pain improvement and in the SF-36 Physical Functioning and Role-Physical domains.

HEALTH-RELATED QUALITY OF LIFE IN HEMOPHILIA B

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OBJECTIVES: To describe health-related quality of life (HRQoL) and health utility of persons with hemophilia B, and to determine the association of these measures with self-reported joint pain and motion limitation. METHODS: The prospective, longitudinal Hemophilia Utilization Study (HUGS-VB), a study involving 2613 hemophiliacs from 80 centers in the United States. Of these, 361 patients with hemophilia B from six U.S. Hemophilia Treatment Centers from June 2009 to September 2010. At initial interview, participants or their parent(s) answered questions regarding demographic and clinical characteristics, HRQoL, health utility and self-reported joint pain and motion limitation. HRQoL measures included Short Form-12 (SF-12) for adults and PedsQL for children. Health utility measures used were EQ-5D (adults) and visual analog scale (VAS). RESULTS: Seventy-seven participants (48% adults) were recruited. Adult mean SF-12 mental (MCS-12) and physical (PCS-12) component scores were 54.3 (±6.13) and 47.1 (±11.1) respectively. Participants with mild/moderate hemophilia (mean=50.4±9.0) had significantly better PCS-12 scores than those with severe hemophilia (mean=42.6±12.4; P<0.0390). Mean EQ-SD and VAS scores were 0.85 (±0.16) and 85.5 (±11.1) respectively, with no significant differences between severity groups. PCS-12 and EQ-SD each negatively correlated with self-reported joint pain (PCS-12r=-0.015; EQ-SDr=-0.0017) and motion limitation (PCS-12r=-0.001; EQ-SDr=-0.0081). Better HRQoL was associated with less severe pain or limitation. Pediatric mean total PedsQL scores were 86.6 (±11.2) with pain and 94.7 (±6.13) without pain and psychosocial functioning summary scores of 92 (±14.9) and 82 (±13.3) respectively. Mean VAS score was 86.6 (±14.3). No significant differences were found between severity groups. PF and VAS scores each negatively correlated with self-reported joint pain (PFr=-0.0127; VASr=-0.0245) and motion limitation (PFr=-0.0009; VASr=-0.0015). CONCLUSIONS: While previous HRQoL studies have examined hemophilia A and its associated clinical aspects, this is the first focusing on the hemophilia B population. As hemophilia A and B may have different clinical manifestations, HRQoL data on hemophilia B can help define disease burden in this group. One limitation is the current small sample size, which will increase as additional participants continue to be enrolled.

MEASUREMENT CHARACTERISTICS OF THE SF-36 IN CHRONIC NEUROMUSCULAR DISORDER

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OBJECTIVES: Quality of life (QoL) remains an important consideration in the care of patients living with chronic neuromuscular disorder (NMD). The factor structure of the SF-36 was evaluated in patients with NMD in order to determine the appropriateness of this instrument to assess QoL in this clinical population. METHODS: Confirmatory factor analyses were conducted on self-report SF-36 data from 245 individuals diagnosed with one of 8 different structural models of the SF-36 were evaluated against data. RESULTS: The underlying factor structure of the SF-36 in NMD was observed to be consistent with contemporary theoretical models of the instrument. The traditional measurement model of SF-36, however, performed comparatively poorly. CONCLUSIONS: The use of the SF-36 in individuals with...