A330

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CONCLUSIONS: The FSS scale is an instrument reliable and valid to measure muscle fatigue in Brazilian patients with myopathy.

PDN68

SOCIAL ECONOMIC BURDEN AND HEALTH-RELATED QUALITY OF LIFE IN PATIENTS WITH RARE DISEASES IN EUROPE (BURQOL-RD PROJECT). METHODS OF SELECTION OF 10 DISEASES FOR A EUROPEAN SURVEY

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OBJECTIVES: The BURQOL-RD project is intended to develop a disease based model of the economic and health-related quality of life (HRQOL) for patients with rare diseases (RD) and their caregivers in Europe. We described the methodology used to select a set of 10 RD to be approached in a pilot study. METHODS: BURQOL-RD project counts with 20 partners, from 8 European countries, Spain, UK, France, Germany, Sweden, Italy, Hungary and Bulgaria. A two-round Delphi process was used to generate consensus in the selection of the 10 RD among the project participants. The wide variability and dispersion of the responses received in the two Delphi rounds of prioritization suggested that an additional procedure should be implemented to improve the representativeness of the selected RD. Carroll’s triangle graphic tool was applied based on the respondents’ determinants. RESULTS: The two rounds of Delphi panel yielded into a prioritised list, to which the Carroll diagram was applied, taking into account three determinants: prevalence, availability of effective treatment and need for care. The final set of RD was obtained to be targeted in the pilot study of BURQOL-RD. Cystic Fibrosis, Prader-Willi Syndrome, Haemophilia, Duchenne Muscular Dystrophy, Epidermolysis Bullosa, Fragile X Syndrome, Scleroderma, Mucopolysaccharidosis, Juvenile Idiopathic Arthritis and Histioctysis. CONCLUSIONS: This methodology permitted to obtain an equilibrated set of RD for the pilot study of BURQOL-RD project. The model that will be generated will not only be suitable to apply in a wide range of RD but it will also be sufficiently flexible to identify and adapt to the challenges faced by the different health and social care systems of EU member states.

Urinary/Kidney Disorders – Clinical Outcomes Studies

PUK1

BELATACEPT VERSUS TACROLIMUS: RESULTS OF A INDIRECT ANALYSIS FROM A SYSTEMATIC REVIEW OF IMMUNOSUPPRESSIVE THERAPIES FOR KIDNEY TRANSPLANT RECIPIENTS

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OBJECTIVES: To systematically identify and summarise the evidence of renal transplant recipients treated with belatacept and other effective options. In particular, comparing tacrolimus, the cornerstone of renal transplantation therapy, with newer therapies that have been introduced since 2003. METHODS: An electronic literature search of MEDLINE, Current Contents and the Cochrane Library was conducted, plus manual reference checks of all articles involving controlled trials of kidney transplants and immunosuppressive therapy between 2003 and July 2010. Studies were assessed for eligibility and quality by two reviewers who extracted data independently. Studies were classified according to (CN) avoidance or reduction, steroid avoidance, and induction therapies. Results were expressed as risk ratio (RR) with 95% confidence intervals (CI). Where necessary, indirect comparison techniques were used to compare different forms of tacrolimus with belatacept. RESULTS: Thirty-five studies from an initial list of 1055 citations were included in the analysis. Results show CN avoidance leads to higher incidence of acute rejection (RR 2.52, 95% CI 1.13–5.75), which is a known predictor for graft loss, but reduced chronic allograft nephropathy. Tacrolimus superior provides better rejection prophylaxis compared with ciclosporin (RR 0.38, 95% CI 0.21–0.70), and ciclosporin produces lower acute rejection compared with belatacept (RR 0.32, 95% CI 0.2–0.55). Indirect analysis showed that tacrolimus is superior to belatacept in acute rejection prophylaxis (RR 0.18, 95% CI 0.08–0.39), but leads to more cases of a decrease in glomerular filtration rate (GFR) (RR 1.37, 95% CI 0.92–2.03); however, the long-term impact of a reduction in GFR in the context of a CN regimen is not clear at present. CONCLUSIONS: Direct and indirect comparisons demonstrate that CNIs, and in particular tacrolimus, remain superior even against more recent compounds for preventing acute rejection. However, more research needs to be done to find the optimum combination of therapies.

Puk2

COMPARATIVE EFFECTIVENESS OF INVESTIGATIONAL COMPOUND FERUMOXITOL FOR THE TREATMENT OF IDIOPATHIC DEFICIENCY ANEMIA IN CHRONIC KIDNEY DISEASE: SYSTEMATIC REVIEW AND MIXED TREATMENT COMPARISON

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OBJECTIVES: To evaluate the therapeutic impact of ferumoxitrol and investigational compound ferumoxytol for the treatment of iron deficiency anaemia (IDA) associated with chronic kidney disease (CKD) compared to alternative iron replacement therapies (IRT). Primary interest was the improvement in haemoglobin (Hb) from baseline levels. METHODS: A comprehensive systematic review was conducted to identify and evaluate the randomized controlled trials investigating the use of ferumoxytol for the treatment of IDA in CKD where efficacy is defined as Hb change from baseline and IRTs included intravenous (IV) and oral treatments. Twelve electronic databases were searched up to November 2010 (language unrestricted). Two reviewers independently assessed each identified reference and conducted the subsequent data extraction. Method quality of each included trial was also independently assessed in accordance with NICE guidelines. A standard meta-analysis comparing oral iron to ferumoxytol was initially conducted, reflecting the trial programme. The full network of evidence that included ferumopxitrol and IRTs was synthesised using a mixed treatment comparison (MTC). RESULTS: Seventeen published trials and one unpublished clinical study provided the heterogeneous trial base for MTC analysis. Ferumoxytol was significantly favoured when compared to oral iron therapy by conventional meta-analysis (0.61, 95% CI: 0.44–0.79, P value < 0.0001) which was supported by the results from the MTC efficacy analysis (0.48, 95% CI: 1.24–2.9). Significant differences in efficacy were not observed between ferumoxytol and any of the alternative IV iron therapies.

CONCLUSIONS: The results from the conventional meta-analysis showed that the model favoured investigational compound ferumoxytol, in terms of increasing Hb, compared to oral iron therapy suggesting a modelled equivalence to currently approved alternative IV iron treatments.

Puk3

SOURCES OF HETEROGENEITY AMONG OVERACTIVE BLADDER CLINICAL TRIAL ESTIMATES OF TOLTERODINE AND FESITORDERINE REDUCTIONS OF URINARY INCONTINENCE EPISODES RELATIVE TO PLACEBO

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OBJECTIVES: To explore potential sources of heterogeneity among estimates of tolterodine (TOL) and fesitorderine (FES) efficacy relative to placebo (PBO) in patients with overactive bladder and urgent urinary incontinence (UUI) from randomized clinical trials (RCTs) published from 2001 – 2010. METHODS: RCTs evaluating TOL 4mg, solifenacin 5mg and/or 10mg, or FES 4mg or 8mg compared to PBO reporting mean reduction of UUI episodes/d from baseline to endpoint were identified. Treatment response (mean PBO responses and FES responses) were tested for heterogeneity using Cochran’s Q statistic. Where heterogeneity was present, other study variables (baseline UUI, baseline micturitions, gender, age, diary evaluation days, publication year, and study duration) were evaluated for potential confounding using linear regression methods. RESULTS: Statistical heterogeneity was found among the 17 PBO responses (mean reduction of UUI) of the included studies. PBO response increased with publication year, which accounted for more than 27% of response variation. Publication year (p < 0.002), gender (p = 0.003), and study duration (6-week vs. other) (p = 0.006) were significant predictors of PBO response (adj. R2 = 0.95). Direct and indirect treatment responses were also heterogeneous. Among the nine TOL trials, treatment responses remained constant over publication year while PBO responses increased, resulting in a net decline in TOL treatment effect (p = 0.0928). The majority of this decline was explained by publication year and study duration (adj. R2 = 0.7039). The four FES 8mg UUI responses also displayed a publication year-dependent decrease leading to a decreasing treatment effect relative to PBO. However, this trend was almost fully predictable by differential baseline UUI episodes (adj. R2 = 0.9721).

CONCLUSIONS: Publication year, gender, 6-week duration, and baseline UUI were found to be significant predictors of PBO response or treatment effect. Additional research should be done to understand why PBO response has increased over time