THE POTENTIAL IMPACT OF CHELATION THERAPY (CT) ON THE QUALITY OF LIFE (QoL) OF PATIENTS WITH IRON OVERLOAD (IO)

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OBJECTIVES: Repeated blood transfusions can result in IO and lead to life-threatening complications in patients with sickle cell disease (SCD), thalassaemia (TLA) or myelodysplastic syndrome (MDS). The most common IO CT, Desferal (DFO), requires infusions of 8–12 hours, 5–7 days per week, potentially limiting QoL and inhibiting adherence. METHODS: A literature review (339 abstracts; 130 articles), analysis of four IO patient transcripts (one MDS, one SCF, two TLA) and interviews with three IO experts were conducted to assess the potential impact of CT on patients’ lives. RESULTS: Results suggested the significant negative impact of CT with DFO on patient QoL, particularly social life, self-image, intimate relationships, emotional well-being, pain and sleep, though very few published studies (15) measured this impact with validated QoL instruments. No IO or CT-specific QoL instruments were found. Published articles and patient/clinician input suggested a need for easier, efficacious and safe oral IO treatments, given the impact of current CT on QoL inhibits adherence. CONCLUSION: Limited empirical studies assessed the impact of CT or IO on QoL, though all reviewed articles stated that CT’s QoL impact is significant and would improve with oral therapy. However, the impacted QoL domains may differ by age, condition, and how long CT has been used. It is recommended to continue both the qualitative and the quantitative study of QoL in chelated patients in patients with MDS, SCD, and TLA using validated instruments in order to further our understanding of the issues and improve patient’s quality of life.

HOW PATIENTS WITH HAEMOPHILIA ARE SATISFIED WITH THEIR TREATMENT

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OBJECTIVES: Treatment of patients with haemophilia, a congenital bleeding disorder, treated with repeated infusions of clotting factor concentrates, requires a substantial amount of economical and human resources and therefore it is mandatory to investigate treatment satisfaction of haemophilic patients. Purpose of this study was to evaluate patients’ experience of different treatment regimens and to validate the newly developed haemophilia-specific treatment satisfaction scale for adults (Hemo-SatA) in the COCHE Study (Cost of Care of Hemophilia Study). METHODS: 233 adult haemophilia patients without inhibitors from 23 Italian Haemophilia Centres were enrolled in the naturalistic, multicenter, longitudinal COCHE Study. Treatment satisfaction was assessed with the Hemo-SatA, which consists of 34 items pertaining to 6 domains (ease & convenience, treatment satisfaction was assessed with the Hemo-SatA, which consists of 34 items pertaining to 6 domains (ease & convenience, efficacy, burden, specialist, centre, general). RESULTS: Mean age of patients was 37 years. Around one-third of the patients received prophylactic treatment (n = 75) and most of them were treated with recombinant products (58.8%). In general patients were satisfied with their treatment. Patients were mostly unsatisfied in the dimensions “ease & convenience” and “efficacy”; 42% worried about the risk of inhibitors associated with their medication and 14% were unsatisfied with the number of infusions that are needed to stop a bleeding. Significant differences in treatment satisfaction (Hemo-SatA) were found for socio-demographic data concerning marital status and age groups (“burden”); separated persons were more unsatisfied with their treatment (“specialist”, “general satisfaction”, “Total Hemo-Sat”). Significant differences were found as well for clinical data concerning ‘treatment type’, ‘type of haemophilia’ and ‘target joints’; patients with more than 1 target joint were more unsatisfied (“efficacy”). Psychometric characteristics of the pilot testing of the Hemo-SatA could be confirmed in the COCHE Study. CONCLUSIONS: Results could confirm that treatment satisfaction is an important outcome criterion in the treatment of patients with chronic diseases. Hemo-SatA proved to be a valid and reliable instrument assessing.

NEUROLOGICAL DISORDERS

EXTERNAL VALIDATION OF THE PROBABILISTIC MARKOV MODEL ESTIMATING THE COST-EFFECTIVENESS OF MEMANTINE VERSUS STANDARD CARE IN ALZHEIMER DISEASE FROM A UK PERSPECTIVE

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A Markov model was developed to estimate the cost effectiveness of memantine compared with no pharmacological treatment in British AD patients with MMSE £14 over a 2-year period. It simulated a patient’s progression through series of health stages combining different level of severity, dependency and setting of care. Transition probabilities for the model were computed on the basis of the data from the LASER-AD cohort (London and the Southeast Region Alzheimer’s Disease) over six months, and were then extrapolated using the Markov assumption to simulate the long-term course of disease. Since then the LASER-AD has been extended and now 18 months data follow up are available. OBJECTIVE: To validate the model externally by comparing the results extrapolated in the model with those observed in the LASER-AD cohort at 18 months. METHOD: A Markov cohort analysis was conducted on the model in order to compute the percentages of patients in the different health stages at all cycles using the distribution at baseline of the LASER-AD. The analysis was conducted on the “standard care” treatment strategy only, and was stopped at the third cycle (18 months). Percentages from the model were compared with those obtained from the LASER-AD study after having observed 95 patients with a baseline MMSE £14. RESULTS: Based on model analyses, after 18 months 84.5% of patients are severe, 87.8% are dependent and 71.5% are institutionalized (versus respectively 83.8%, 90.3% and 73.3% in the LASER-AD). The difference between the two estimates is greatest for mortality (respectively 40.6% versus 31.6%). CONCLUSION: It is rare to have the opportunity to validate a pharmacoeconomic model externally. These analyses show very similar estimates of the disease course between the memantine UK pharmacoeconomic model and the ‘real’ long-term data from the LASER-AD cohort. This strengthens the modeling approach used.

COST-EFFECTIVENESS OF ADDING MEMANTINE TREATMENT TO PATIENTS RECEIVING STABILISED DOSES OF DONEPEZIL IN THE UK

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OBJECTIVES: Assess the cost-effectiveness in a UK setting of providing memantine treatment to moderate to severe
Alzheimer’s disease (AD) in patients receiving stable donepezil treatment compared with not providing memantine. METHODS: Data from a U.S. multicenter randomised clinical trial that compared memantine versus placebo in moderate to severe AD patients on stable doses of donepezil was used to evaluate the cost effectiveness of providing memantine to donepezil. Using methodology developed by Kurz et al., patients were classified at each visit as dependent or non-dependent according to their ADCS-ADL19 scores. Mean weekly costs were estimated from the National Health Service and Personal Social Services perspectives for patients with MMSE £14 that participated in a UK epidemiological study—the LASER-AD study. QALYs per dependency were estimated from a Danish Study. Per week mean acquisition cost and effectiveness of treatment were £975, 0.6311 and £288; 0.3207 for dependent and non-dependent patients. Total drug costs for the 24-week period were £492 for donepezil and £937 for memantine plus donepezil. Costs, QALYs and time of non-dependence were associated with each dependency level and added to obtain total outcomes over the 24-week study period. RESULTS: Over the evaluation period, memantine added to patients stabilised on donepezil was associated with an additional 0.0112 QALYs, an additional 1.77 weeks of independence and a £771 cost reduction compared with donepezil alone. The cost reduction is not statistically significant but suggests that clinical advantages offset some of the cost of adding memantine. CONCLUSION: This analysis suggests that memantine treatment provided to patients receiving stable donepezil treatment is cost-effective compared with not adding memantine. As costs and QALYs were assessed retrospectively, further prospective studies are required to support this finding.

PNL3

RETROSPECTIVE COMPARATIVE ANALYSIS OF ANTIDEMENTIA MEDICATION PERSISTENCE PATTERNS AT 3 YEARS IN SPANISH ALZHEIMER DISEASE PATIENTS TREATED WITH DONEPEZIL, RIVASTIGMINE, GALANTAMINE AND MEMANTINE

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OBJECTIVE: To determine persistence of treatment with donepezil (DON), rivastigmine (RIV), galantamine (GAL) and memantine (MEM) in patients with Alzheimer Dementia (AD) in a Spanish population setting. METHODS: Retrospective AD cohort study performed in nine Primary Care Health Centers from four different Autonomous Communities (Andalucía, Cantabria, Cataluña & Valencia) in Spain. Descriptive standard analyses were performed. ANOVA and Chi-square tests were used to show the differences among mean duration therapy and treatment adherence after 52 weeks. A Kaplan-Meier survival analysis was applied to assess overall pattern persistence after three-year of follow up, and the log rank test was used for testing significance. RESULTS: A total of 299 patients (44.8% female), mean age 77.88 years (SD: 6.32), were included; 101 DON (34%), 103 RIV (35%), 51 GAL (17%) and 42 MEM (14%). Mean treatment duration was slightly longer and significant for DON patients 83.3 weeks (95% CI: 77.2–93.9) than for the other drugs: RIV [56.1 weeks (36.1–76.2), GAL [56.7 weeks (41.1–72.3)] and MEM [52.1 weeks (35.2–69.1)]. Log Rank = 10.16, p = 0.017. CONCLUSION: This retrospective study including Spanish AD patients showed numerically differences on treatment adherence after one year of therapy among the four antidementia medications commercially available. The global treatment persistence during the three-year follow up was significantly higher in patients treated with donepezil compared to those who received rivastigmine, galantamine or memantine.

PNL4

THE SOCIAL AND ECONOMIC BURDEN OF PAEDIATRIC EPILEPSY IN IRELAND: A PROSPECTIVE STUDY

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OBJECTIVE: To estimate the overall burden of pediatric epilepsy on the family and Irish health care system and to establish whether there is a relationship between epilepsy profile and this burden. METHOD: The sample was drawn from a population of children with epilepsy attending a tertiary pediatric neurology clinic in Dublin. Data was collected prospectively on health care resource use and time lost from school and work. Diary cards were completed at three monthly intervals. RESULTS: Complete data was available on 127 children aged 15 months to 16.7 years (median 8.8), 54% were male and 52% lived in a rural setting. A total of 61% had cryptogenic or symptomatic epilepsy, 63% had partial seizures and 53% had frequent seizures (>10 seizures/month). The annual cost of epilepsy was significantly higher for those with cryptogenic/symptomatic epilepsy (£9248) and frequent seizures (£9145) relative to idiopathic epilepsy (£2600) and no/in frequent seizures (£3951) (P < 0.0001). Children with frequent seizures had a higher risk of being hospitalised (P = 0.03) and lost more days at school (P < 0.0005). 50% of families contacted the pediatric liaison nurse, and 12% made more than 5 contacts. Five percent of children attended their GP while 37% of families had independently sought complementary medicine. CONCLUSIONS: The economic and social burden of pediatric epilepsy is substantial and relates to the epilepsy syndrome and frequency of seizures. In this prospective study a large dependence on epilepsy liaison nurse support was found, an area that requires extra resources. A large number of families also sought advice from non medical sources.

PNL5

A PROSPECTIVE STUDY ON THE IMPACT OF A CHILD’S EPILEPSY ON THEIR QUALITY OF LIFE AND THEIR FAMILY

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OBJECTIVES: The aim of this prospective study was to determine the relationship between seizure type, seizure frequency and epilepsy syndrome on the quality of life of both the child and family over a one year period. METHOD: The sample was drawn from a population of children with epilepsy attending a tertiary Paediatric Neurology service in Dublin. Data was collected on seizure type and frequency, epilepsy syndrome, physi-