PHM8

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), thallasaemia (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 (539 abstracts; 130 articles), analysis of four IO patient transcripts (one MDS, one SCDA, 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.

PHM9

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 Centers 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, 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

PNL1

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.

PNL2

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