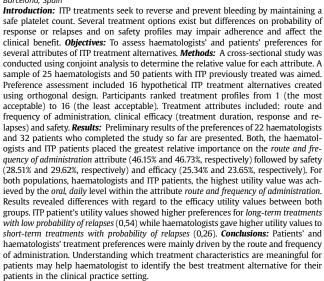
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hospitals were collected. Double bootstrap data envelopment analysis was utilized to assess the technical efficiency of the centres where an input-oriented variable return to scale model was adopted. Bootstrap truncated regression was later conducted to identify the factors affecting the performance levels. Results: The average biascorrected technical efficiency score was found to be 0.62 (SD=0.15). 13.3% thalassaemia treatment centres demonstrated good efficiency scores (0.8-1.0). Presence of multidiscipline specialized treatment team is found to positively influence the efficiency levels. In contrary, having longer operating hours did not improve the performance levels. Conclusions: This study provides primary baseline performance benchmark for public hospitals with thalassaemia treatment centres in Malaysia. The findings could also provide vital insights for policymakers and service managers for better resource allocation to ensure optimal healthcare delivery to thalassaemia patients in Malaysia.

### Systemic Disorders/Conditions - Patient-Centered Research

# PATIENTS' AND HAEMATOLOGISTS' PREFERENCES ON IMMUNE THROMBOCYTOPENIA (ITP) TREATMENT ALTERNATIVES: A CONJOINT ANALYSIS





## **HEALTH STATE UTILITIES ASSOCIATED WITH SICKLE CELL DISEASE AND ITS TREATMENT: LITERATURE REVIEW**

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Objectives: Sickle cell disease (SCD) typically requires lifelong comprehensive care and is associated with early mortality. The only available potentially curative therapy is allogeneic hematopoietic stem cell transplant with a matched sibling donor, recommended only for younger patients and limited by donor availability. As new treatments are introduced, cost-utility analyses (CUA) are required to understand their value. To conduct CUAs, appropriate and robust health state utility estimates are required. The purpose of this study was to review utilities representing SCD and its treatments. Methods: Two PubMed literature searches were conducted: (1) publications mentioning SCD and utilities or related terms; (2) publications on costutility analyses of treatments for SCD so that reference sections could be searched for additional sources. Abstracts were reviewed, and potentially relevant articles were obtained to assess eligibility. Information extracted from each eligible article included study type, utility assessment methods, sample characteristics, health states represented by utilities, and limitations. Results: The two searches yielded 124 and 24 abstracts, respectively. Eight of these references were relevant to SCD utilities. Three presented EQ-5D values for SCD. However, applicability of these values in CUAs is questionable because the populations had specific treatment characteristics that may not be generalizable to most patients. Also, the extent to which this generic instrument captures the burden of SCD and its treatment options is unknown. Two other references included CUAs based on estimates provided by two or three physicians, rather than utilities from patients or a preference-based task. Another two CUAs used utilities representing treatment approaches instead of SCD. The final article was a literature review with no additional primary sources of

utilities. Conclusions: Utilities representing SCD are limited. Given the prevalence of this condition, utility values are needed to represent various aspects of the disease state in economic models examining the value of new treatments.

### PSY44

## **ECONOMIC BURDEN OF MYALGIC ENCEPHALOMYELITIS/** CHRONIC FATIGUE SYNDROME (ME/CFS) TO PATIENTS: **COMPARATIVE STUDY**



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Objectives: Research is performed in framework of COST (European Cooperation in Science and Technology) Action 15111 EUROMENE (European Myalgic Encephalomyelitis/Chronic Fatigue Syndrome (ME/CFS) Research Network) to investigate the opportunities for evaluation of economic impact of ME/CFS to patients. Methods: To achieve the objectives of this research, a study based on patient-reported survey has been carried out in Latvia, Italy and the United Kingdom (UK). The survey included questions concerning the socio-economic consequences of the disease, particularly regarding the health care costs. For data processing and analysis, the methods of economic analysis and statistical analysis are embraced. Results: In Latvia the survey has been launched with coverage of 100 patients (with dominance income of €500 per household member monthly). The results show that 1% of respondents spend more than €100 as an out-pocket payment for medicines and health care services monthly, 1.8% spend €51-100 for medicines, health care services and food supplements monthly, 17.2% spend €21-50, and 80% of respondents spend until €20 monthly to reduce the ME/CFS consequences. The study performed in Italy with 87 participants suffered by ME/CFS found that 23% were unemployed and 55% had an income less than €15.000 annually. Patients spend average €210 monthly on medication and therapy. A study conducted in the UK covered 262 patients with ME/ CFS and healthy controls. The analysis shows marked lower economic well-being of people with ME/CFS in comparison with healthy controls. Average adjusted income for participants with CFS/ME was £12.242, but for healthy controls - £23.126. Considering the prevalence of ME/CFS in the UK population, the total cost of illness was estimated at £1.713 million per year. Conclusions: The patient-reported outcomes is a significant tool to collect the data for evaluation of socio-economic impact of ME/CFS, but for comparative studies the purchasing power parities should be taken into account.

# PSY45

# PATIENT-REPORTED OUTCOMES USED IN CLINICAL TRIALS OF SYSTEMIC LUPUS ERYTHEMATOSUS: A REVIEW OF SUITABILITY FOR FDA LABELLING CLAIMS



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Objectives: This review sought to identify patient-reported outcome (PRO) instruments that have been used in clinical trials of systemic lupus erythematosus (SLE) and examine their suitability for FDA labelling claims, based on proximity of the concept they measure to symptoms of SLE and evidence of content validity in SLE patients. Methods: A search was conducted on clinicaltrials.gov to identify PROs used as primary and/or secondary endpoints in SLE clinical trials. A literature review of identified PROs was then conducted, examining their structure, the concept they measure and evidence of content validity as per FDA standards, including involvement of SLE patients in instrument development. Results: 49 different PRO instruments, used across 129 trials, were identified. The majority (39) measured multi-dimensional constructs or distal concepts of disease, including healthrelated quality of life (HRQOL), impacts on mental function (depression, anxiety, stress, anger), cognition, physical activity, sleep, social functioning and work performance, as well as self-management/efficacy, coping strategies and global impression of change. 10 identified PROs measured proximal concepts/ symptoms of disease, namely fatigue and pain. Out of all 49 instruments that were identified, only 4 PROs, all HRQOL instruments, were SLE-specific and three of those, the LupusQoL, LupusPro and SLEQOL, had satisfactory evidence of content validity. A further review of these three instruments revealed that only LupusQOL contained domains that measure well-defined and proximal concepts/symptoms of disease, namely pain and fatigue. Conclusions: Despite a considerable number of PROs that have been used in SLE clinical trials, only three instruments had satisfactory evidence of content validity in SLE patients in line with FDA standards. Only one of these instruments, the LupusOOL, contains domains measuring proximal concepts/symptoms of disease (pain and fatigue) that could be considered suitable for inclusion in endpoint models in SLE drug development with intended PRO-based labelling claims in the US.

# WORK PRODUCTIVITY LOSS IN PATIENTS WITH **INFLAMMATORY ARTHRITIS**

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