PSY99

A PREDICTIVE MODEL TO INCLUDE UNCERTAINTY IN MEAN HEALTH UTILITIES ESTIMATION: IMPACT OF SOCIAL CLASS, BODY MASS INDEX AND CHRONIC DISEASES

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OBJECTIVES: Including second order uncertainty for utility values when building cost-effectiveness modelling is challenging due to its odds distribution. Our approach enables including uncertainty related to the effect of sociodemographic characteristics or chronic diseases on mean utilities included as input in different models.

METHODS: In Spain, the EQ-5D-5L was incorporated in the National Health Survey carried out between June 2011 and June 2012. The data of the survey were used to develop a Spanish Statistical Webpage. The following factors could be considered in this study: sex, age, and social class (3 categories), body mass index from self-perceived height and weight and EuroQol 5D questionnaire. For the estimation of the mean utilities we divided the process in two steps. First, a two component regression model was used to estimate the mean utility value. In addition, using Cholesky decomposition of the variance-covariance matrix uncertainty on these parameters could be included in a mathematical model. RESULTS: This study included 20209 Spanish people, mean age 51.3 years, 53.0% females. All mean values were significantly lower in obese compared to normal weight in both sexes and social classes. Men with mental health diseases and women with stroke showed the highest disutility values. CONCLUSIONS: This methodology enables estimating mean utility values based on individual characteristics of social class, body mass index and chronic diseases and its associated uncertainty. This way is possible to include more exhaustive second order uncertainty in mean utility values for probabilistic sensitivity analysis in cost-effectiveness modelling.

PSY99

WORK PRODUCTIVITY AND IMPAIRMENT AMONG PATIENTS WITH LIGHT CHAIN Amyloidosis

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OBJECTIVES: Light chain (AL) amyloidosis is a rare disease characterized by misfolded protein deposits in tissues and vital organs. This study examines whether health-related quality of life (HRQoL) is associated with productivity and impairment of patients with AL amyloidosis. METHODS: An online survey was administered to patients with AL amyloidosis (N = 341) from the United States, Europe, and other countries to assess HRQoL and work impairment. The Work Productivity and Activity Impairment: Specific Health Problem (WPAI:SHP) questionnaire was used to assess disease-related Absenteeism, Presenteeism (i.e. impairment at work); and Productivity Loss (i.e., overall work impairment) among employed patients (n=108). Employed patients were classified into two groups (“No impact” = 0% “Impact” > 0%) based on their scores for each WPAI scale. HRQoL was assessed with the SF-36v2® Health Survey Physical and Mental Component Summary scales (PCS, MCS), and the Medical Outcomes Study (MOS) Sleep Scale-6R. Cross-sectional associations between HRQoL and the dichotomous WPAI impairment measures were analyzed using separate multivariable logistic models. Odds ratios were interpreted in terms of 5 point decrements in HRQoL scores. RESULTS: Based on multivariable logistic regression models, high HRQoL scores were associated with all WPAI outcomes (p < 0.05). While associations between PCS and each WPAI outcome were fairly similar the impact of low PCS score was more than doubled the odds of Productivity Loss (OR=2.19) than MCS (OR=2.06), whereas, a five-point lower PCS score had a slightly lower impact (80% greater odds than MCS). While associations between MCS and each WPAI outcome were fairly similar, the impact of low MCS score was the most closely related to Presenteeism (IRR) were interpreted in terms of 5 point decrements in HRQoL scores. CONCLUSIONS: While associations between PCS and each WPAI outcome were fairly similar the impact of low PCS score was more than doubled the odds of Productivity Loss (OR=2.19) than MCS (OR=2.06), whereas, a five-point lower PCS score had a slightly lower impact (80% greater odds than MCS). Work Productivity and Activity Impairment: Specific Health Problem (WPAI:SHP) utilities which were equal to 1 based on individual characteristics. For those with not perfect health state the mean utility value was estimated using generalized linear regression analysis. In combination, this two regression models allowed to estimate the mean utility value. In addition, using Cholesky decomposition of the variance-covariance matrix uncertainty on these parameters could be included in a mathematical model. RESULTS: This study included 20209 Spanish people, mean age 51.3 years, 53.0% females. All mean values were significantly lower in obese compared to normal weight in both sexes and social classes. Men with mental health diseases and women with stroke showed the highest disutility values. CONCLUSIONS: This methodology enables estimating mean utility values based on individual characteristics of social class, body mass index and chronic diseases and its associated uncertainty. This way is possible to include more exhaustive second order uncertainty in mean utility values for probabilistic sensitivity analysis in cost-effectiveness modelling.

PSY100

HEALTH-RELATED QUALITY OF LIFE AND RATES OF EMERGENCY ROOM VISITS AND HOSPITALIZATION IN PATIENTS WITH AL AMYLOIDOSIS: A PROSPECTIVE ANALYSIS

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OBJECTIVES: Light chain (AL) amyloidosis is a rare disease represents considerable personal impact. The associated societal costs including anxiety, depression and feelings of guilt associated with hereditary diseases. Social impacts for caregivers included reduced social participation, limited social support networks, and affected relationships with partners, other children and family members. Caregivers also reported experiencing limited expert disease knowledge from healthcare professionals. The reduced ability to work was a common area of concern for caregivers as were the financial impacts associated with caring for an individual with a rare disease. Caregivers own physical health is impacted due to the direct impacts of caring for an individual with a rare disease. As a result, the indirect impact the burden contributes to the overall cost of an individual’s disease. Estimates within the literature of the monetary impact of caring for an individual with a rare disease ranges up to 31% of the total cost of illness. CONCLUSIONS: Caring for an individual with a rare disease can produce significant impact on the family. These costs contribute to the overall cost of illness. This information is important when considering the benefits of new treatments but also the cost and value of these treatments.

PSY101

RARE DISEASES, ARE CAREGIVERS JUST AS AFFECTED AS PATIENTS?

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OBJECTIVES: Research on rare diseases from the patient perspective is limited; however, research into the impact on parental caregivers of affected individuals is even more limited. The aim of this study was to gather evidence to explore the impact of caring for an individual with a rare disease. METHODS: An inaugural literature review was conducted to identify studies exploring the impact of caring for somebody with a rare disease. Evidence from these studies was used to establish an overview of the impact of living with an individual(s) with a rare disease. RESULTS: The published studies reviewed focused on many rare diseases. Common themes emerged across the disease areas representing multiple domains of quality of life (QoL). Caregivers reported considerable emotional impacts including anxiety, depression and feelings of guilt associated with hereditary diseases. Social impacts for caregivers included reduced social participation, limited social support networks, and affected relationships with partners, other children and family members. Caregivers also reported experiencing limited expert disease knowledge from healthcare professionals. The reduced ability to work was a common area of concern for caregivers as were the financial impacts associated with caring for an individual with a rare disease. Caregivers own physical health is impacted due to the direct impacts of caring for an individual with a rare disease. As a result, the indirect impact the burden contributes to the overall cost of an individual’s disease. Estimates within the literature of the monetary impact of caring for an individual with a rare disease ranges up to 31% of the total cost of illness. CONCLUSIONS: Caring for an individual with a rare disease can produce significant impact on the family. These costs contribute to the overall cost of illness. This information is important when considering the benefits of new treatments but also the cost and value of these treatments.

SYSTEMIC DISEASES/CONDITIONS – Health Care Use & Policy Studies

PSY104

REGIONAL VARIATIONS IN APPRAISAL AND UPTAKE OF NEW TREATMENTS FOR ULTRA RARE DISEASES IN THE UK: A CASE STUDY OF ATALuren FOR NONSENSE MUTATION DUCHENNE MUSCULAR DYSTROPHY (NMDMD)

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OBJECTIVES: NICE Highly Specialised Technology guidance only applies to England, which represents 80% of the UK population. We describe regional variations in gaining reimbursement for the estimated 70 nMMD UK patients eligible for ataluren. METHODS: We review the nMMD experience in England, Wales, and NI and the Isle of Man. Detailed desiers were submitted to NICE and SMC following different templates and requirements.EMA conditional marketing authorisation was granted in July 2014. Over a year later, the first patient received NHS-funded ataluren in Scotland via an Individual Patient Treatment Request. Individual funding requests elsewhere in the UK were unsuccessful. Following patient and political lobbying over a 13-month