

OBJECTIVES: The objective of this study was to assess the cost-effectiveness and cost-utility of dasatinib versus FLAM (fludarabine, cytarabine, mitoxantrone) chemotherapy in treatment of adult patients with Ph+ ALL with resistance or intolerance to prior therapy from the public payer's perspective in Poland. **METHODS:** The constructed Markov model compared the costs and health effects of dasatinib and FLAM treatment for the average patient in the life-time horizon. The model included the following health states: survival without progression, survival after allogeneic hematopoietic stem cell transplantation (allo-HSCT), survival after progression and death. Data on clinical effectiveness of dasatinib and FLAM were retrieved from single-arm clinical trials, whereas data for overall survival after allo-HSCT and after progression was taken from long-term studies. The utilities for all health states were identified by systematic review. The following costs from public payer's perspective were considered: substances and their administration (chemotherapy), monitoring, allo-HSCT, monitoring and chemotherapy after allo-HSCT and palliative care. Discount rates of 5% for costs and 3.5% for benefits were used. **RESULTS:** The amount of life years gained associated with dasatinib arm and FLAM arm in the life-time horizon was 1.79 LYG and 1.17 LYG, respectively. Treatment with dasatinib resulted in 1.48 QALY and with FLAM - 0.94 QALY. The incremental cost-effectiveness ratio (ICER) of dasatinib versus FLAM was estimated to be €24,145/LYG from the perspective of Polish National Health Fund and it is below the threshold accepted for Poland (€27,275/QALY). The result of cost-utility analysis are almost equal to the threshold accepted for Poland and amount to €28,146/QALY. **CONCLUSIONS:** Taking into account the status ALL as ultra-rare disease and the results of economic evaluation, dasatinib is the cost-effective strategy in comparison with FLAM chemotherapy in treatment Ph+ ALL patients with resistance or intolerance to prior therapy from the public payer's perspective in Poland.

PSY76

ASSOCIATION BETWEEN FEEDING TYPES AND IRON STATUS IN INDIA

Prieto Patron A, Detzel P

Nestlé Research Center, Lausanne, Switzerland

OBJECTIVES: The introduction of complementary foods around the age of six months is necessary as infant's need for energy and nutrients starts to exceed what is provided by breast milk. One of the key nutrients that infant's demand could not be met only by partial breastfeeding is iron. The objective of this study is to shed some light on the role of complementary foods on the iron status of children using the food categories reported in the Indian National Family Health Survey 2005-06 (NFHS-3). **METHODS:** The analysis presents the results of three sets of regressions to associate hemoglobin / anemia levels and 17 feeding categories controlling for cofounders. First, ordinary least square (OLS) regressions on the hemoglobin level shown. Second, logistic regressions were used to estimate the odd ratio of becoming anemic and moderate or severe anemic. Finally, with proportional odds (ordered logit) model it was estimated the risk from passing to one to other category in anemia. **RESULTS:** "Commercial fortified baby food" together with "partial breastfeeding" and "infant formula" having the stronger positive and statistical significant association with iron status for infants aged six to 23 months. Additionally, "fruits rich in vitamin A" and "meat and fish" have also a significantly positive association with iron status. On the contrary, "tea or coffee", "no commercial porridge" and "bread or noodles" categories tend to have a significant negative association with iron measurement. **CONCLUSIONS:** Infants in India are introduced relatively late to iron rich foods which would allow them to replenish their iron stores from birth at the appropriate moment. Infant nutritional guidelines should emphasize the benefits of fortified baby food as well as a variety of food that would enhance the iron status on this highly vulnerable age group.

PSY77

RETROSPECTIVE COHORT STUDY USING DATA FROM THE UK CLINICAL PRACTICE RESEARCH DATALINK AND HOSPITAL EPISODE STATISTICS TO ASSESS UNPLANNED HOSPITALISATION IN PATIENTS WITH MULTIPLE MYELOMA

Raluy-Callado M¹, Lambrelli D¹, DeCosta L², Gonzalez-McQuire S³

¹Evidera, London, UK, ²Amgen Ltd., Uxbridge, UK, ³Amgen (Europe) GmbH, Zug, Switzerland

OBJECTIVES: Understanding resource use in multiple myeloma (MM) is important for assessing the value of new treatments. This study investigated unplanned hospitalisations and factors associated with increased incidence of hospitalisation in patients with MM. **METHODS:** Primary care data from the UK Clinical Practice Research Datalink and Hospital Episode Statistics were collected during 1 January-31 December 2010 for adults who were alive and already diagnosed with MM on 1 January 2010. The primary outcome, unplanned hospitalisation, was defined as an emergency admission involving an overnight stay. Patient and disease characteristics were reported according to hospitalisation status and number of hospitalisations. **RESULTS:** At baseline, median age of the 769 patients was 72 years and median time since diagnosis was 3.1 years. 31.6% of patients had at least one unplanned hospitalisation; of these, 43.2% were rehospitalised during the study period. Patients with unplanned hospitalisations were older (median 74 vs 71 years) and more likely to have renal failure (43.6% vs 31.2%) or cardiac disease (40.3% vs 29.1%) than those without hospitalisation ($p < 0.005$). There were no significant differences in the prevalence of diabetes or prior stem cell transplantation. Admissions were most frequently to general medicine (35.7%) or haematology (19.8%) departments. Excluding MM, hospitalisations were most commonly for acute lower respiratory infection (6.6%) and lobar pneumonia (5.4%). Stays were longer for patients with at least two unplanned hospitalisations than for those with one (mean 12 vs 10 days; $p = 0.0761$). **CONCLUSIONS:** Renal failure and cardiac disease (common among elderly MM patients) were significantly associated with unplanned hospitalisations. Admissions were often lengthy and required treatment in specialist units (e.g. haematology departments). Such hospitalisations are likely to incur high costs. The results indicate an unmet need in MM management to more successfully control the burden of the disease and thereby reduce associated resource utilisation.

PSY78

INPATIENT BURDEN AMONG PATIENTS WITH CYSTIC FIBROSIS WHO ARE HOMOZYGOUS FOR THE F508DEL MUTATION

Hodgkins P¹, Ayyagari R², Guo J², Wagener J³, O'Sullivan AK¹

¹Vertex Pharmaceuticals, Boston, MA, USA, ²Analysis Group, Inc, Boston, MA, USA, ³University of Colorado, Aurora, CO, USA

OBJECTIVES: To examine inpatient utilization among patients with cystic fibrosis (CF) and homozygous for the F508del CFTR gene mutation. **METHODS:** Medical chart data from patients with CF ≥ 12 years old were collected in France, Germany, Italy, Spain, Australia and Canada. Demographics and clinical characteristics were obtained for a 12-month baseline period and a follow-up period ranging from 2-36 months. Proportions of patients hospitalized, hospitalization rates, and length of stay were assessed overall and by age (12-17, ≥ 18 years), lung function (percent predicted forced expiratory volume in 1 second [ppFEV1] $\geq 70\%$, 41-69%, $\leq 40\%$), and country. **RESULTS:** Data for 523 patients were included. Baseline mean \pm SD age was 24.8 ± 9.5 years and mean \pm SD ppFEV1 was $67.1 \pm 22.9\%$. Over a mean of 27 months follow-up, 19% of patients had 1 hospitalization, 11% had 2, and 37% had ≥ 3 . The mean \pm SD rate of hospitalizations was 1.2 ± 1.5 per patient-year. In the follow-up period, the proportion of patients with at least 1 hospitalization and the rate of hospitalization were highest for the severe ppFEV1 group relative to moderate and mild groups (86%, 73%, and 55% and 2.1 ± 2.0 , 1.4 ± 1.6 and 0.7 ± 1.1 per patient-year respectively). The overall mean length of stay was 10.7 ± 7.5 days; it was 9.2 ± 6.8 days for the mild lung function group vs 11.0 ± 6.7 days for the severe group. The hospitalization rate ranged from 0.6 ± 1.1 per patient-year in Spain to 1.7 ± 1.9 in Australia; trends by lung function were consistent across countries. **CONCLUSIONS:** Patients with CF and homozygous for the F508del CFTR gene mutation have high rates of hospitalization. Hospitalization rate varies by country, but is consistently higher for patients with the lowest lung function, congruent with a progressive disease.

SYSTEMIC DISORDERS/CONDITIONS - Patient-Reported Outcomes & Patient Preference Studies

PSY79

TRANSTHYRETIN FAMILIAL AMYLOID POLYNEUROPATHY IMPACT ON HEALTH-RELATED QUALITY OF LIFE

Inês M¹, Coelho T², Conceição I³, Ferreira LN⁴, Carvalho M³, Costa J⁵

¹Instituto de Medicina Molecular, Lisboa, Portugal, ²Unidade Clínica de Pamiloidose, Hospital de Santo Antonio, Porto, Portugal, ³Centro Hospitalar de Lisboa Norte, Lisbon, Portugal, ⁴Universidade do Algarve, Faro, Portugal, ⁵Institute of Molecular Medicine, Lisbon, Portugal

OBJECTIVES: Transthyretin Familial Amyloid Polyneuropathy (TTR-FAP) is a rare, progressive, debilitating and life-threatening neurodegenerative disease. The purpose of this study was to assess the health-related quality of life (HRQoL) impairment of TTR-FAP disease versus Portuguese general population. Literature on TTR-FAP patients HRQoL is scarce at worldwide level and no evidence on HRQoL index score for Portugal has been published. **METHODS:** HRQoL was measured using the validated EuroQoL five dimensions three levels (EQ5D-3L) questionnaire being the index score (utility) calculated through the Portuguese scoring algorithm. The Portuguese general population reference set ($n = 1500$) was pooled with TTR-FAP patients specific data ($n = 1091$) extracted from Transthyretin Amyloidosis Outcomes Survey (THAOS) registry. Demographic variables include gender and age. Ordinary Least Squares (OLS) regression for utility was set to test if being asymptomatic carrier caused HRQoL impairment, conditional in other individual characteristics. Generalized linear models (GLM) were specified for disutility in order to disentangle and quantify TTR-FAP effect on HRQoL versus Portuguese general population. Akaike information criteria (AIC) were used to select the most adequate statistical model. **RESULTS:** In a scale from -0.50 to 1.00 the average utility score was 0.76 (0.25) for general population, 0.823 (0.24) for TTR-FAP asymptomatic carriers ($n=525$) and 0.50(0.37) for symptomatic TTR-FAP patients ($n=566$). OLS including independent gender and age variables, indicated no significant statistical effect on utility for being a TTR-FAP asymptomatic carrier (p -value 0.54) versus general population. GLM (AIC -0.58) detected a significant statistical effect for gender, age and being symptomatic TTR-FAP patient. Average women aged 44 years and symptomatic TTR-FAP patient, has a 40% impairment on utility versus women aged 44 years from general population. **CONCLUSIONS:** The preference-based utility measures used in this study adequately disentangle TTR-FAP disease impact on patient's health-related quality of life. This study allows us to quantify the large HRQoL effect that TTR-FAP induces.

PSY80

HEALTH STATE UTILITIES FOR GAUCHER DISEASE TYPE 1

Ganz ML¹, Stern S², Ward AJ¹, Selzer M³, Nalysnyk L³, Hamed A³, Weinreb N⁴

¹Evidera, Lexington, MA, USA, ²Evidera, Bethesda, MD, USA, ³Zenzyme, a Sanofi Company, Cambridge, MA, USA, ⁴University Gaucher Disease - Fabry Disease Treatment Center, Coral Springs, FL, USA

OBJECTIVES: Economic evaluations of Gaucher disease type 1 (GD1) performed to date have used utilities for narrowly defined GD1 health states derived from specific patient populations. The aim of this study was to calculate alternative utilities for GD1 health states based on a standard disease severity measure. **METHODS:** Health states were based on components of the Disease Severity Scoring System (DS3), a validated measure capturing hematologic, visceral, and bone domains of GD1. Nine states were defined using combinations of DS3 severity categories (mild, moderate, marked, and severe) and the presence or absence of bone pain (BP) or skeletal complications (SSC): mild, mild+BP, mild+SSC, moderate, moderate+SSC, marked, marked+SSC, severe, and severe+SSC. DS3 and quality of life (SF-36) data came from a sample of GD1 patients enrolled in the ICGG Gaucher Registry (275 observations, 101 patients). SF-36 data were converted to UK EQ-5D utilities using published methods. We fitted a generalized estimating equation, accounting for multiple observations per patient, containing terms for DS3 severity categories, BP,