Abstracts

Patients with MT of any age, on ICT for at least three years, were sequentially enrolled at eight Italian Thalassemia Care Centers. HRQoL was measured in >14 year-old, patients, who completed 2 generic instruments, EQ-5D and Short Form-36 (SF-36). RESULTS: Results refer to 121 patients, with median age = 29.8 (14.1–48.5), 49.6% male. At enrolment 87.6% of patients had at least one thalassemia related complication, 48.3% were treated with Deferoxamine, 32.5% treated with Deferiprone, 19.2% were treated with both; 14.0% changed treatment regimen at least once during the observational period (11.6 median months). EQ-5D profile patients reported moderate problems with “mobility” (9.1%), “self care” (0.8%), “usual activities” (23.5%), moderate or severe “pain/discomfort” (60.5%) and “anxiety/depression” (39.5%). The EQ-Visual Analogue Scale had a mean = 73.0 (median = 75.0, from 30 to 100). Similar trends were observed with the SF-36 ones: in particular the mean + SD Physical Component Summary score was 47.7 ± 8.4; the mean ± SD Mental Component Summary score was 45.1 ± 8.8. CONCLUSIONS: Thalassemic patients have impaired levels HRQoL: both physical and mental components seem to be compromised from the disease or ICT and related consequences. Therapies improving patients’ satisfaction with and compliance to ICT may have positive consequences not only on clinical effectiveness but also on overall patient’s wellbeing.

DEVELOPMENT AND SCORING OF THE SATISFACTION WITH IRON CHELATION THERAPY INSTRUMENT FOR PATIENTS WITH IRON OVERLOAD

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OBJECTIVES: Patients with thalassemia, sickle cell disease (SCD), and myelodysplastic syndromes (MDS) require infusion iron chelation therapy (ICT) involving 8–12 hour infusions, 5 days per week, potentially limiting quality of life (QoL) and inhibiting adherence in patients already limited by their condition. Thus, satisfaction with ICT is an important treatment outcome. To date, there is no well-established measure to quantify patient satisfaction with ICT. The aim of this study is to describe the development and scoring of a treatment satisfaction instrument for patients taking ICT. METHODS: Based on a literature review, and patient and clinician interviews, a 28-item instrument was developed as funded by Novartis. This included an assessment of: satisfaction with prior experience with ICT; satisfaction with ICT characteristics; adherence to treatment; preferences; and behavioural intentions. U.S. and U.K. patients with thalassemia, SCD, or MDS (n = 110) currently taking ICT completed the satisfaction instrument. The scoring of the instrument and the assessment of its reliability and validity was performed on the 19 satisfaction items. The 9 items related to past experience, intentions and preferences were scored separately. RESULTS: The Principal Component Analysis using a varimax rotation revealed a four dimensional structure that explained 63% of the total variance. The factors of the Satisfaction with ICT instrument were labelled: Perceived Effectiveness of ICT (6 items); Burden of ICT (5 items); Acceptance of ICT (5 items); and Side-effects of ICT (3 items). Internal consistency reliability for all subscales was good, with alpha coefficients ranging between 0.80 for Acceptance of ICT to 0.86 for Perceived Effectiveness. CONCLUSIONS: Preliminary analyses suggest that the Satisfaction with ICT instrument is reliable. Further validation of the instrument is required to assess its test-retest reliability, construct validity and responsiveness. The instrument could be used in routine clinical practice or in clinical trials to measure satisfaction with ICT.

PHM12

PHYSICIANS’ PREFERENCES TOWARD COAGULATION FACTOR CONCENTRATES IN THE TREATMENT OF HEMOPHILIA PATIENTS WITH INHIBITORS: A DISCRETE CHOICE EXPERIMENT

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OBJECTIVE: Treatment for hemophilia patients with inhibitors is costly and challenging for its complexity, without clear agreement on drug selection or optimal dosing regimen for the first-line management of bleeding episodes. This study sought to identify treatment attributes that are important to hematologists in the United States. METHODS: A conjoint analysis was conducted to elicit preferences using a discrete choice experiment. Twelve attributes were assessed: risk of human viral infections, possibility that the titer of the inhibitor may rise, reduction in the likelihood of dose-related thromboembolic events, the number of infusions required to stop hemorrhage, infusion preparation time, infusion time, infusion volume, time required to stop bleeding, time required to alleviate pain, prophylaxis use, ability to undergo major surgery, and cost of medications. Thirty hematologists completed questionnaires involving twelve choice tasks with trade-offs between three scenarios (most likely to use, no preference, and least likely to use). Data were analyzed using a multinomial logit model to obtain relative importance of each attribute. RESULTS: Responding hematologists (with an average of 13 years of experience treating hemophilia patients with inhibitors) treat on average a total of 28 patients including four inhibitor patients per month. “Time required to stop bleeding” was the most important factor affecting treatment decisions (relative importance (RI) = 16.3%). Physicians also preferred treatment products that possessed quick pain relief (RI = 12.9%), no possibility that the titer of inhibitor may rise (RI = 12.8%), fewer number of infusions required to stop a hemorrhage (RI = 12.7%), and absence of risk of human viral infection (RI = 10.8%). CONCLUSIONS: The study revealed the most important attributes of treatment for hemophilia patients with inhibitors from the physician perspective. Future studies should compare physician preferences with those of hemophilia patients with inhibitors.

SATISFACTION WITH IRON CHELATION THERAPY AND ITS IMPACT ON ADHERENCE IN PATIENTS WITH BETA THALASSEMIA MAJOR: RESULTS FROM THE ITHACA STUDY

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Patients with β-Thalassemia Major (MT) require life-long blood transfusions, which often cause iron overload that, if left untreated, may increase patients’ morbidity and mortality. Iron Chelation Treatment (ICT), is based on 8–12 hour infusions of Deferoxamine for 5–7 days/week and/or Deferiprone orally administered. ICT aims to reduce iron overload but low satisfaction and low compliance, lead to potentially negative consequences on treatment effectiveness. OBJECTIVES: To investigate ICT satisfaction in MT patients and explore its relationship with their thinking about stopping medication. METHODS: The
Italian-THAlassemia-Cosy-&-Outcomes-Assessment (ITHACA) was a naturalistic multicentre study conducted to evaluate costs, quality of life, compliance and treatment satisfaction in MT patients undergoing ICT. In total 126 MT patients >12 years old completed a 28-item satisfaction instrument consisting of 4 domains (perceived effectiveness, burden, side effects, acceptance. Response continuum ranged from 1 (very dissatisfied) to 5 (very satisfied). RESULTS: The median age was 29.4 years (12.3–48.5), with an equal proportion of males and females. At enrolment, 48.0% were treated with Deferoxamine, 33.6% with Deferinprope and 18.4% with combination treatment. 86.5% of patients had at least one MT-related complication and 13.5% changed treatment regimens at least once during the observational period (11.6 median months). Mean satisfaction scores for “acceptance”, “side effects”, “burden” and “perceived effectiveness” were 3.37, 3.57, 3.87 and 4.29 respectively. Simple linear regression analysis showed that satisfaction with “acceptance” (R² = 11%), “burden” (R² = 13%) and “side effects” (R² = 12.3%) are significantly and positively associated with the adherence item “never thinking about stopping medication” (p < 0.0001 in both cases). CONCLUSIONS: The level of satisfaction with “burden”, “acceptance” and “side effects” in MT patients undergoing ICT can help to predict patient adherence. Strategies able to improve satisfaction with ICT are expected to increase patients’ adherence, with potential significant consequences on treatment effectiveness.

**PHM14**

**PATIENT PREFERENCE AND WILLINGNESS-TO-PAY FOR ANTICOAGULANT TREATMENT OPTIONS IN PATIENTS RECEIVING ORAL ANTICOAGULANT TREATMENT (OAT): A DISCRETE CHOICE EXERCISE**

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OBJECTIVES: New anticoagulant drugs, alternative to vitamin K antagonist (VKA) are currently under clinical evaluation. Patients’ preferences should be considered in the development of new therapeutic strategies. Objective of this study was to elicit patients’ preferences on different treatment options. METHODS: A discrete-choice-exercise (2 different scenarios in 9 pair-wise comparisons) was applied to consecutive patients of all ages on ICT. The questionnaire was completed by 237 patients (55% male; mean age = 63.3 ± 14.41% on-stable-OAT). A significant monetary discrimination was reached for all attributes, except “interactions” and “dose adjustment”. The conditional-probit model demonstrated that patients are willing-to-pay (WTP) per month: €79 for once-daily administration-tablets vs one subcutaneous weekly-injection; €41 for once-daily administration-tablets vs twice-daily administration-tablets; €21 for once-monthly vs twice-monthly; €18 for each 6 month vs once-monthly visits; €23 for a drug without bleeding-risk. The model showed important differences between stable and starting OAT patients: the WTP for less frequent monitoring and no interactions is greater for patients in stable-OAT; the WTP to avoid bleedings is greater for starting patients. Also the employment status matters; employed patients are more willing to pay for a treatment with less frequent monitoring. CONCLUSIONS: To our knowledge, this study is the first to elicit preferences from patients in OAT. The importance of this study is the achievement of patient’s preferences in a simply and well accepted method to allow planning optimal health care.

**INDIVIDUAL’S HEALTH**

**PHI1**

**BUDGET IMPACT ANALYSIS OF AVODART (DUTASTERIDE) IN THE TREATMENT OF BENIGN PROSTATIC HYPERPLASIA (BPH) IN POLAND**

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OBJECTIVES: To estimate impact of dutasteride reimbursement on budget from the perspective of National Health Fund (NHF) in Poland. METHODS: Decision-analytic model was developed to assess budget impact of dutasteride. Costs and resource use are from published literature and NHF catalogue of procedures. Costs of drugs, surgical intervention and prostate cancer treatment were taken into account. Clinical data, including complications and prostate cancer rates, were obtained from the clinical effectiveness analyses of dutasteride. Budget impact analysis was performed in four different scenarios with regard to reimbursement reference price and rates (Scenario 1: 50% reimbursement rate, reference price = reference price for Penetrex; Scenario 2: 50% reimbursement rate, reference price = Avodart price; Scenario 3: 70% reimbursement rate, reference price = reference price for Penetrex; Scenario 4: 70% reimbursement rate, reference price = Avodart price). RESULTS: In case of Avodart (dutasteride) reimbursement, NHF would save: 65,888–562,612 PLN in year 1, 197,664–1,687,836 PLN in year 2, 300,477–3,212,590 PLN in year 3 and 376,229–3,212,590 PLN in year 4 and later, depending on assumed reimbursement reference price and rate. CONCLUSIONS: Reimbursement of dutasteride would result is savings from the perspective of the National Health Fund in Poland.

**PHI2**

**HEALTH ECONOMIC CONSEQUENCES OF THE CHOICE OF FOLLICLE STIMULATING HORMONE ALTERNATIVES IN IVF TREATMENT**

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OBJECTIVE: The choice is between two types of hormones used for follicle stimulation in IVF treatment—recombinant FSH (rFSH) and the urine-derived menotrophin. A literature review by NICE in the UK carried out in 2004 documented that the two types of hormones were equally effective and safe consequently it was recommended to use the cheaper menotrophin. Based on findings by the European and Israeli Study Group (EIGS), the objective was to analyse the health economic consequences of this choice between the two types of hormone in IVF treatment in Denmark. METHODS: Based on the EIGS study a prospective cost-effectiveness analysis, which compared menotrophin (Menopur®) and rFSH (Gonal-F®), was carried out. The analysis had a health care sector perspective with the inclusion of costs of hormone stimulation, visits, ultrasound, IVF, ICSI and inpatient stay at the hospital. Danish unit costs (2005) were applied. Differences in costs were compared with differences in effects to