months. RESULTS: 137 patients were enrolled (median age = 28.3 years, from 2.7 to 48.5 years, 49.6% male). At enrolment 112 (81.7%) patients had at least one thalassemia related complication (42.3% had hepatic, 58.4% endocrine, 18.2% cardiac diseases, 55.5% viral infections). Patients treated with DFO were 51.5%, 31.6% were treated with L1, 16.9% with DFO + L1. Treatment in patients taking DFO cost on average €552.88/patient/month plus €211.20 for pump and consumables; patients taking L1 cost €383.25/patient/month, patients taking DFO + L1 cost €918.41. Overall, mean direct cost was €1245.33/patient/month, with ICT representing 55.4% of costs, followed by transfusions (33.1%), hospitalizations and surgery (3.3%), laboratory and instrumental tests and medical visits (3.1%), concomitant medications (1.6%), non-medical costs (transportation, 3.4%). CONCLUSION: Transfusion and ICT account for 90% of total costs, corresponding to approximately €1000/patient/month. These results can be considered conservative because some sources of costs (e.g. nursing, home care) were not estimated, anyway we do not expect the amount of not included costs would significantly affect our results.

**PHM8**

**INDIRECT COSTS OF BETA THALASSEMIA MAJOR: RESULTS FROM THE ITACA STUDY**

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People with hematological disorders such as beta thalassemia major (TM) who regularly receive blood transfusions need iron chelation therapy (ICT) to prevent iron overload. The drugs most frequently given are deferoxamine and deferoxiprone. So far, little is known about the costs related to iron chelation treatment. OBJECTIVE: To receive information about indirect costs due to ICT. METHODS: The Italian-THAlassemia-Cost-&-Outcomes-Assessment (ITHACA) is a naturalistic, multicenter, retrospective study involving patients with TM of any age, on ICT for at least 3 years, sequentially enrolled at 8 Italian Thalassemia Care Centers. Indirect costs were estimated with modified versions of the “Health and Labour Questionnaire” (HLQ). RESULTS: A total of 116 Italian TM patients completed the HLQ. Sixty-one patients (52.7%) were in paid employment. 21 (34.4%) had experienced absenteeism from work due to ill health in the previous 2 weeks. The average absenteeism among patients in paid employment was 0.7 days/week. Indirect costs related to absenteeism are 27.55 euros per patient a week (based on net earnings). Fifteen patients (26.4%) of patients in paid employment) were hindered by health problems at work. Indirect costs per patient based on hours needed to catch up on tasks neglected due to health problems were 9.3 euros/week. Patients in our study received 1.24 hours of household help/week, adding up to 64.74 hours per patient a year. No differences were detected in the above between patients using deferoxamine, deferoxiprone or a combination of both, possibly due to the limited sample size. 21 children (or their caregivers) completed the “HLQ children-part” (average age 10.7). In the previous twelve weeks 18 children missed schooldays (mean = 0.64 days/week) and 6 children missed regular activities. CONCLUSIONS: TM and current treatment strategies have a high impact on patients’ productivity leading to considerable costs. Children with TM miss school days on a regular basis.

**PHM9**

THE EVALUATION OF ECONOMIES OF SCOPE IN GENETIC SCREENING BY DNA TECHNOLOGY: A MODELING STUDY

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OBJECTIVE: To assess by a modeling example under which conditions a combination of adult population genetic screening programs is cost-effective. METHODS: Based on a systematic literature review, hereditary haemochromatosis (HFE) was selected as a potentially cost-effective primary DNA screening target. A secondary condition where population screening exhibits favourable effectiveness yet unfavourable cost-effectiveness is hereditary nonpolyposis colorectal cancer (HNPCC). A decision tree for DNA screening for selected mutations in adult male Caucasians was constructed from a health care sector perspective for HFE, HNPCC, both combined and all three alternatives including first-degree relatives. Cost data from a recent HFE screening cost study in Germany were applied; HNPCC mutation prevalences were estimated based on data from the German HNPCC consortium. RESULTS: The model renders a cost-effectiveness ratio of approximately 100.000 Euro per life-year gained for the dominated HNPCC screening option, 300 EUR/LYG for HFE screening and 600 EUR/LYG for combined screening. Cost-effectiveness is most sensitive to different values for HFE penetrance and cost of genetic counselling found in the literature. Despite high incremental cost-effectiveness ratios for single HNPCC mutations added, the average cost-effectiveness ratio of the DNA test remains moderate if high test specificity is assured. Effectiveness and cost-effectiveness improve substantially if first-degree relatives are included. CONCLUSIONS: Rather than disease predisposition screening for frequent single nucleotide polymorphisms (SNPs) with low penetrance, it appears worthwhile to screen for rare mutations with high penetrance, combined with screening for HFE. Product developers in the in-vitro diagnostics industry may follow this approach to design cost-effective screening tools like DNA chips. The screening algorithm should be designed in a way to minimize the number of false-positives. Pre-test counselling should identify cases of familial cancer and ensure that negative test results are not misinterpreted as private mutations cannot be identified by a test for known HNPCC mutations.

**PHM10**

QUALITY OF LIFE IN PATIENTS WITH BETA THALASSEMIA MAJOR: RESULTS FROM THE ITACA STUDY

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OBJECTIVES: Patients with β-Thalassemia Major (MT) require lifelong blood transfusions, which often cause iron overload that may increase patients’ morbidity and mortality. Iron Chelation Treatment (ICT), based on 5–12 hour infusions of Deferoxamine for 5–7 days/week, and/or Deterfiprone orally administered, is aimed to reduce iron overload but can be related to low satisfaction, low compliance and potentially negative consequences on clinical effectiveness and patients’ wellbeing. Aims: to investigate on Health-Related Quality-of-Life (HRQoL) of MT patients under ICT. METHODS: The Italian-THAlassemia-Cosy-SC-Outcomes-Assessment (ITHACA) was a naturalistic multicentre study conducted to evaluate costs, HRQoL, compliance and treatment satisfaction in MT patients undergoing ICT.
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 THALASSEMA 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