OBJECTIVES: To assess cost-effectiveness of PXF, used in combination with G-CSF versus standard mobilization options used alone, to enhance mobilization of haematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation (auto-HCT). Target population, as defined by Polish opinion leaders, are patients with NHL/HL or MM whose cells mobilize poorly, i.e. predicted poor mobilizers (peripheral blood CD34+ cell count <10/mL) and proven poor mobilizers (failure of previous mobilizations). **METHODS:** PXF is an add-on therapy to currently reimbursed standard mobilization (SM) options: G-CSF or chemotherapy plus G-CSF. Two scenarios were compared: with and without PXF. Analysis was performed using a Markov decision model, developed in *TreeAge Pro 2013*. A probabilistic (Monte Carlo simulation) model with time-dependent transition probabilities included initial cycle, during which the individual undergoes mobilization and auto-HCT if cell collection is successful, and four mutually exclusive health states: progression-free survival (remission and durable remission – lymphomas only), further treatment after progression, palliative treatment/observation and death. A one-year Markov cycle length was used with half-cycle correction. Baseline cohort characteristics, disease progression and utility estimates were obtained from systematic literature review and questionnaire study among Polish clinical practitioners. The analysis was conducted from the Polish public payer, patients, and societal perspective, over a lifetime horizon. Discount rates were 5% (costs) and 3.5% (outcomes). **RESULTS:** The mean QALY gain were 7,378 (PXF) and 6,452 (SM). The mean costs were 188,404 PLN (€45,019) (PXF) and 157,073 PLN (€37,532) (SM). Base-case incremental cost-utility ratio (ICUR) in NHL/HL/MM population was 33,821 PLN/QALY (€8,082). Probability of PXF being cost-effective in Poland when compared to SM is 99,8% (current threshold of 105,801 PLN/QALY (€25,281)). **CONCLUSIONS:** Based on accepted cost/QALY threshold values in the Polish settings, PXF was proved to be cost-effective option for NHL/HL/MM patients with poor response to SM regimens.

PSY43

COST-UTILITY ANALYSIS (CUA) OF BELIMUMAB (BEL) IN THE TREATMENT OF ADULT PATIENTS WITH ACTIVE, AUTOANTIBODY-POSITIVE SYSTEMIC LUPUS ERYTHEMATOSUS (SLE)

Walczak J[1], Kopel J[1], Sołtys E[1], Dziurda D[2]

[1]*Arcana Institute, Cracow, Poland,* [2]*GSK, Warszawa, Poland*

OBJECTIVES: To estimate the cost-utility of BEL treatment of patients with SLE as an add-on therapy to intensive standard of care (SOC) vs. SOC in Poland. **METHODS:** The general population considered in the analysis were patients with SLE with significant disease activity, who met criteria of SLE diagnosis and were ineffectively treated with SOC. CUA was performed from the public payer perspective. The lifetime horizon was assumed. The decision model was developed in *MS Excel* and adjusted to Polish conditions. The calculations were performed using Monte Carlo microsimulation. The model included data from international clinical studies and Polish data on costs and resource utilization. Direct medical costs were included: costs of drugs, administration, diagnostic, monitoring and *costs related to disease activity*. Results were calculated for two patients populations: target population 1 (TP1) - patients with positive anti-dsDNA, low complement and ≥10 scores in SELENA-SLEDAI scale and target population 2 (TP2) - patients with positive anti-dsDNA, low complement, ≥6 scores in SELENA-SLEDAI scale and necessity of corticosteroids use. **RESULTS:** CUA results showed that BEL compared to SOC is more costly however, also more effective therapy. The ICUR was 113,986 PLN/QALY and 108,744 PLN/QALY, respectively for TP1 and TP2. Obtained results are placed slightly above the Polish acceptability threshold. Additional health effects related to BEL treatment instead of SOC were noticed both in LYG and QALY (0.432 and 0.4 LYG; 0.322 and 0.294 QALY, respectively for TP1 and TP2). Moreover, the model suggests lower frequency of cardiovascular events, and also pulmonary and renal complications in BEL arm. **CONCLUSIONS:** Since there is currently no effective treatment option of SLE in Poland, reimbursement of belimumab will give patients an access to a safe and effective therapy, which allows them to return to active life and career and also to improve their quality of life.

PSY44

COST-UTILITY ANALYSIS OF DEFERASIROX FOR THE TREATMENT OF IRON OVERLOAD DUE TO FREQUENT BLOOD TRANSFUSIONS IN THE CHILDREN AND ADOLESCENTS

Walczak J[1], Sołtys E[1], Obrzut G[1], Laczewski T[2]

[1]*Arcana Institute, Cracow, Poland,* [2]*Novartis Poland Sp. z o.o., Warsaw, Poland*

OBJECTIVES: To estimate the cost-utility of deferasirox (DSX) treatment of iron overload due to frequent blood transfusions in children and adolescents in Polish conditions. **METHODS:** Clinical effectiveness data were taken from previously performed systematic review of deferasirox for the treatment of pediatric patients (age ≤ 18 years) with iron overload due to frequent blood transfusions. The way of administration of chelation therapy (parenteral vs. oral) significantly affects patients' functionality and quality of life. Therefore it was decided to perform a cost-utility analysis. Analogous to other economic studies, it was assumed that patient survival was the same for both compared interventions: deferasirox and deferoxamine (DFO). In the analysis a simple decision model was developed. The calculations were performed using Monte Carlo microsimulation technique (100,000 trials). Only direct medical costs were included in the analysis: costs of drugs and their administration, costs of monitoring and costs of blood transfusions. The time horizon of the analysis was one-year period. Two perspectives were considered: a public payer (National Health Fund, NHF) and the patient and NHF perspective. The measure of effects was QALY (quality adjusted life years). **RESULTS:** The results showed that DSX compared to current standard treatment of iron overload due to frequent blood transfusions (DFO) in the children and adolescents is more costly however also more effective therapy from both considered perspectives. The ICUR (incremental cost-utility ratio) of replacing DFO by DSX was 26,180 PN/QALY from NHF perspective. The results from both patient and NHF perspective was similar. **CONCLUSIONS:** With reference to the acceptability threshold in Poland the oral chelation therapy in the population of children and adolescents with iron overload due to frequent blood transfusions with deferasirox is cost effective intervention when compared with deferoxamine.

PSY45

COST-EFFECTIVENESS OF SAPROPTERIN VERSUS PHENYLALANINE FREE DIET IN PATIENTS WITH PHENYLKETONURIA IN EGYPT

ElSisi G[1], Elmahdawy M[1], Abaza S[2], Shalakani A[3]

[1]*Central Administration for Pharmaceutical Affairs, Cairo, Egypt,* [2]*Roche, Cairo, Egypt,* [3]*Ministry of Health, Cairo, Egypt*

OBJECTIVES: Phenylketonuria (PKU) is an orphan disease with incidence rate 1:5000 in Egypt. Cost-effectiveness of Sapropterin versus Phenylalanine (PHE) free diet in PKU patients from the insurer perspective was evaluated over a time horizon of 10 years. **METHODS:** A cohort Markov chain model with six health states: healthy, mild PKU, controlled mild PKU, classical PKU, controlled classical PKU and death was identified based on the process of the disease. The length of a cycle was set at one year. The transition probabilities were derived from updated, previously published studies in Egyptian patients with PKU. Relative risk of Sapropterin and utilities were derived using international published sources. Direct Medical costs were obtained from the Ministry of Health mandatory Tarrif in Egypt. All costs and effects were discounted at 3.5% annually. All costs were reported in Egyptian pounds of the financial year 2013. Deterministic sensitivity analysis was conducted. **RESULTS:** Total costs for Sapropterin and PHE free diet were 304.1687 EGP and 188.6498 EGP respectively. QALYs for Sapropterin and PHE free diet were 0.00566 and 0.00547 respectively. The incremental cost-effectiveness ratio (ICER) for Sapropterin versus PHE free diet was 602,933 EGP/QALY. Sapropterin is not cost effective because it is more than 3 times GDP/capita in Egypt (57,566 EGP). The ICER was most sensitive to the utility of the states 'classical PKU' and 'controlled classical PKU'. **CONCLUSIONS:** World Health Organization recommends that interventions that cost more than 3 times GDP/capita for one Disability Adjusted Life Year (DALY) avoided should not be reimbursed. Despite the difference between DALY and QALY, one can assume they are similar to be able to put a value on the outcome. Sapropterin doesn't represent a good value for money compared to PHE free diet in the Egyptian PKU patients.

PSY46

A COST-UTILITY ANALYSIS OF LIGHTERLIFE TOTAL AS A TREATMENT FOR OBESITY IN THE UNITED KINGDOM

Lewis L[1], Taylor M[2]

[1]*York Health Economics Consortium, University of York, York, UK,* [2]*York Health Economics Consortium, York, UK*

OBJECTIVES: LighterLife Total (LLT) is a weight loss programme for the obese (BMI ≥30kg/m2) combining a very low calorie diet (VLCD) with weekly support groups for behaviour modification. This study evaluated the cost-effectiveness of LLT compared with dietary and surgical interventions. This is the first study assessing the cost-effectiveness of a VLCD. **METHODS:** A cohort model was developed to assess the reduction in BMI in the first 12 months following intervention with LLT, gastric banding, gastric bypass, Weight Watchers, Counterweight, Slimming World and no treatment, and the subsequent yearly BMI increase. Published all cause-mortality was applied by age and BMI. Co-morbidity prevalence (diabetes, colorectal cancer, CHD) was applied by BMI. Costs were applied for each intervention and for co-morbidities, from a UK health care perspective. Utilities were calculated by BMI with an additional decrement for co-morbidities. A 10 year time horizon was used. Analyses were run for two subgroups: BMI 30-40 in and BMI >40. **RESULTS:** BMI 30-40: compared against no treatment, Counterweight, Weight Watchers and Slimming World, LLT was associated with higher costs, but also greater QALYs and was cost-effective against all, with incremental cost-effectiveness ratios of £14,937, £16,004, £16,182, and £19,840, respectively. BMI >40: compared against no treatment LLT incurred higher costs, but also greater QALYs and was cost-effective (ICER = £5,349). LLT was less effective than banding and bypass. The budget impact of uptake of LLT across the UK was assessed for both BMI 30-40 and BMI >40 groups. **CONCLUSIONS:** BMI 30-40: LLT was more costly than dietary interventions, but lead to increased QALYs and was estimated to be cost-effective. BMI >40: LLT resulted in a lower initial BMI reduction than gastric banding and bypass. LLT was estimated to be cost-effective against no treatment in both groups.

PSY47

FULL COST OF PLASMA FROM VOLUNTARY NON REMUNERATED DONORS IN ITALY

Zaniolo O[1], Povero M[1], Gandini G[2], Aprili G[2], Eandi M[3]

[1]*AdRes HE&OR, Turin, Italy,* [2]*University of Verona, VERONA, Italy,* [3]*University of Torino, Torino, Italy*

OBJECTIVES: In Italy, within the legal mandate to pursue national self-sufficiency of secondary blood products, the Regions are starting to organize trade to offset need/availability unbalances. Therefore the determination of the full cost to the Regions of plasma collection and handling is needed. **METHODS:** Plasma is obtained from voluntary, non-remunerated donors either from full blood or from apheresis, and is collected either directly in facilities of the Regional Health Services, or indirectly through the net of donors' associations before being delivered to the transformation industry for processing. Amount and costs of materials and activities needed for collecting, producing, validating, and distributing plasma were obtained from the transfusional medicine department of the Verona province, Veneto Region. Attributable overhead expenses are assumed at 15% of direct cost. When plasma is collected as part of the whole blood or from multicomponent apheresis, common costs are attributed basing on the commercial value of single components, taken as proxy of the willingness-to-pay for them. In an alternative scenario, only product-specific costs are attributed to plasma recovered from whole blood donations, for which the driving need is the supply of red blood cells. **RESULTS:** Total cost per liter of plasma sent for processing is estimated in 114 and 286 euros, respectively, for collection from whole blood and apheresis. Given the current mix of plasma origin, the weighed mean cost of plasma to the Regions before processing charges is estimated in 157 €. When plasma recovered from whole blood donations is considered by-product, its cost per liter sent to industry is estimated in 27 €, and the corresponding weighed mean cost in 92 €. **CONCLUSIONS:** The Italian donor-based system, in addition to

its ethical and social values, supplies plasma at a lower or comparable cost than commercially available.

PSY48

INDIRECT COSTS OF SYSTEMIC LUPUS ERYTHEMATOSUS-RELATED ABSENTEEISM IN POLAND: AN ANALYSIS BASED ON SOCIAL INSURANCE INSTITUTION DATA

Golicki D[1], Karczewicz E[2], Zalewska H[2], Dziurda D[3], Gryglewicz J[3], Gierczynski J[3]
[1]*Department of Experimental and Clinical Pharmacology, Medical University of Warsaw, Warsaw, Poland,* [2]*Department of Statistics and Actuarial Forecasts, Social Insurance Institution (ZUS), Warsaw, Poland,* [3]*Institute of Health Care Organizations, Lazarski University, Warsaw, Poland*

OBJECTIVES: To estimate indirect costs of systemic lupus erythematosus (SLE) in Poland, based on absenteeism in the workplace data derived from Department of Statistics of the Social Insurance Institution (ZUS). **METHODS:** Available insurance information consisted of data on: (1) sick leaves, (2) short-term inability to work - on the basis of decisions authorizing rehabilitation services (3) long-term inability to work – on the basis of medical certificates awarded because of incapacity for work. To calculate indirect costs we used three parallel alterations of the human capital approach (HCA) method - based on: the average monthly gross earnings, Gross Domestic Product (GDP) per capita or gross value added per 1 employee (adjusted by a factor of marginal productivity of labor). **RESULTS:** In 2010, in patients with SLE in Poland, sick leaves, short-term and long-term inability to work were responsible for: 1897, 596 and 27 012 months of absenteeism, in 1600, 112 and 2481 persons, respectively. The total number of 2459 years of lost productivity corresponded to indirect costs of: 100,421,579 PLN, 97,215,041 PLN or 161,743,804 PLN, based on average earnings, GDP per capita or adjusted gross value added per employee, respectively. **CONCLUSIONS:** Two of the three approaches, in addition - the most frequently mentioned in the literature, indicated the indirect costs of systemic lupus erythematosus in Poland at around 100 million PLN per year. Our estimates of indirect costs may be undervalued because it did not include the cost of lost productivity due to premature mortality in the course of SLE, and the costs associated with a reduction in the efficiency of the work done despite of the disease (preseenteism).

PSY49

CHARACTERIZING DISEASE BURDEN IN AN ULTRA-RARE DISEASE IN THE UNITED STATES: TRANSTHYRETIN (TTR) AMYLOIDOSIS PATIENTS & CAREGIVERS

Stewart M[1], Loftus J[2], Lenderking WR[3], Murphy BR[3], Alvir J[4], Roberts L[5], Shaffer S[5], Cicchetti MJ[6], Gleeson S[5]
[1]*Pfizer, Inc, Groton, CT, USA,* [2]*Pfizer Ltd., Tadworth, UK,* [3]*Evidera, Lexington, MA, USA,* [4]*Pfizer, Inc., New York, NY, USA,* [5]*Evidera, Bethesda, MD, USA,* [6]*Pfizer, Inc., Groton, CT, USA*

OBJECTIVES: TTR amyloidosis, a progressive, degenerative ultra-rare genetic disease, can cause familial amyloid polyneuropathy (TTR-FAP) and cardiomyopathy (TTR-CM), requiring substantial caregiver support. This study evaluated the burden of illness on patients' and caregivers' work productivity, health care resource use (HCRU), and health-related quality of life (HRQoL). **METHODS:** An online survey including the Work Productivity & Activity Impairment (WPAI) questionnaire, EQ-5D, & HCRU questions recruited TTR-FAP and TTR-CM patients and caregivers through two U.S.-based patient advocacy groups. **RESULTS:** Thirty-three TTR patients (26 males) and 18 caregivers (7 males) completed the survey. Most were aged over 60; mean disease duration was approximately 6 years (patients) or 5 years (caregivers with disease). Most patients and caregivers had a college degree. Generally caregivers (77.8%) were the primary caregiver for their patient; 61.1% also had amyloidosis. Unemployment was high in patients with TTR-FAP (42.9%), TTR-CM (60.0%), both TTR-FAP/CM (71.4%); only 33.3% of caregivers reported working part/full-time. Employment was highest for TTR-FAP patients (n=10), yet 11.8% missed work, 32.2% were impaired at work and 38.5% reported overall work impairment due to TTR. Liver transplant, the primary treatment option, occurred in 42.4% patients and 18.2% caregivers with disease. A majority of patients reported outpatient visits to health care providers in the past 3 months for disease: 85.7% TTR-FAP, 100% TTR-CM, and 85.7% for TTR-FAP/CM. Hospitalization rates ranged from 14.3-30.0% across all patient groups, with 14.3-23.8% for emergency visits. EQ-5D Index scores for patients were 0.80 (SD=0.14) with transplant, and 0.68 (SD=0.16) without transplant. Caregivers with disease and transplant had lower EQ-5D Index scores (M=0.14, SD=0.35) than those without transplant (M=0.41, SD=0.32). The pattern was similar for EQ-5D VAS results for patient and caregiver groups. **CONCLUSIONS:** TTR amyloidosis is associated with substantial disruption in employment rates, work productivity, high levels of resource use, and poor HRQoL for patients and caregivers.

PSY50

RESOURCE CONSUMPTION EVALUATION ASSOCIATED WITH RITUXIMAB ADMINISTRATION IN PORTUGAL

Pereira C[1], Santos A[2]
[1]*Roche Farmacêutica Química, Lda., Amadora, Portugal,* [2]*Prime Focus Health, Paço de Arcos, Portugal*

OBJECTIVES: Determine the costs associated with rituximab intravenous (iv) preparation and administration in follicular non-Hodgkin lymphoma (NHL) and estimate the difference *versus* rituximab subcutaneous (sc) formulation, considering material resources (MR) consumption and health care professionals (HCP) time spent in each procedure. Patient's and chair time savings in hospital Day Care Unit (DCU) were also estimated. **METHODS:** Rituximab iv data was collected, between November 2012 and January 2013, through face to face interviews with pharmacists and DCU nurses responsible for the preparation and administration in each hospital. The HCP time cost was calculated by multiplying their income per hour by the average time spent on each procedure; MR costs were determined based in official databases or in "table values" provided by the manufacturers. Rituximab sc administration time was based in the respective pivotal clinical trial - SABRINA (BO22334). **RESULTS:** Ten hospitals from mainland Portugal were included, with a weekly average of 7 NHL patients treated with rituximab iv. The HCP average overall active time spent with rituximab iv preparation and administration, per treatment cycle, was about 89 minutes *versus* 16 minutes estimated for sc. An average overall cost reduction of 93% was estimated with sc *versus* iv (3€ *versus* 45€, respectively). DCU chair time capacity could be increased by 3 and 7 fold if one considers combination or maintenance therapy, respectively, with rituximab sc *versus* iv, due to SC much faster administration. Rituximab sc reduces the overall time patients spend in an infusion chair by 95% (7 min with sc *vs.*143 min with iv). **CONCLUSIONS:** Rituximab sc formulation potentially offers significant resource (material and HCP time) savings, improves hospital organization and provides clear benefits for patients regarding time saved and administration convenience. Ultimately, rituximab sc increases hospital efficiency that's critical in the current economic climate.

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

PSY51

THE ASSOCIATION BETWEEN SEVERITY OF 'AVERAGE' PAIN (NPRS) AND THE EQ-5D INDEX IN PATIENTS WITH NEUROPATHIC PAIN

Chambers C[1], Odeyemi I[2], Currie C[3], Poole CD[3]
[1]*Astellas Pharma Europe Ltd., Staines, UK,* [2]*Astellas Pharma Europe Ltd., Staines, UK,* [3]*Cardiff University, Cardiff, Wales, UK*

OBJECTIVES: Pain is an important driver of health-related utility. Our purpose was to characterise the association between pain severity and the EQ-5D index. **METHODS:** Paired values for the Numerical Pain Rating Scale (NPRS) average pain score (previous 24 hours) and the EQ-5D index were available from a prospective, non-interventional study of people with neuropathic pain treated with an 8% capsaicin patch (Qutenza™). The NPRS records pain on an integer scale between 0 and 10 units, representing no pain and worst imaginable pain, respectively. The EQ-5D index is derived from impairment level (none/moderate/severe) across five domains (Mobility, Self-care, Usual activities, Pain & discomfort, Anxiety & depression), and values health-related utility on a scale of 1 to 0, meaning perfect health and death, respectively. Generalized linear mixed models with a normal probability distribution, identity link function, and a first-order autoregressive covariance structure were tested to determine the relationship between EQ-5D index score (scale) and NPRS average 24 hour pain score (ordinal). **RESULTS:** For the purposes of this preliminary analysis, 170 patients with NP contributed 353 combined observations from baseline observation and follow-up assessments at week-8 and week-12. The GLMM model that best fitted the data (smallest Information criterion) had one random effect (subject + intercept) and one fixed effect (NPRS + intercept). The fixed-effects coefficients were: (Intercept) 0.728 + (NPRS1: β 0.000; 95%CI -0.0186, 0.186) + (NPRS2: -0.045;-0.205,0.116) + (NPRS3: -0.075;-0.227,0.078) + (NPRS4: -0.207;-0.364,-0.049) + (NPRS5: -0.181;-0.338,-0.024) + (NPRS6: -0.315;-0.471,-0.159) + (NPRS7: -0.323;-0.478,-0.167) + (NPRS8: -0.458;-0.618,-0.299) + (NPRS9: -0.638;-0.825,-0.451) + (NPRS10: -0.740; -0.927,-0.553). Predicted utility was highly correlated (R^2=0.753) with observed utility. Mean squared error for predicted utility was 0.033 (sd 0.052). **CONCLUSIONS:** Neuropathic pain was highly correlated with utility with a difference of around 0.8 utility units across the NPRS range. All domains of the EQ-5D differed across the NPRS.

PSY52

MEASURING PROS THAT MATTER TO BARIATRIC AND BODY CONTOURING SURGERY: THE BODY-Q

Pusic A[1], Cano S[2], Scott A[1], Tsangaris E[3], Klassen A[3]
[1]*Memorial Sloan-Kettering Cancer Center, New York, NY, USA,* [2]*ScaleReport, Stotfold, United Kingdom,* [3]*McMaster University, Hamilton, ON, Canada*

OBJECTIVES: Health care payers are interested in funding bariatric surgery because it resolves a range of obesity-related health problems. However, following weight loss, many patients are left with unsightly excesses of skin and require body-contouring surgery. Our team has developed a new PRO instrument (i.e., the BODY-Q) to measure satisfaction and quality of life of bariatric and body-contouring surgery patients. Unlike existing PRO instruments, the BODY-Q is composed of scales that measure appearance-related concerns, which is an important reason why patients seek treatment. The BODY-Q also stands apart as it the only PRO instrument designed to measure change in patients concerns throughout the entire weight loss journey. **METHODS:** We followed international guidelines for the development of a PRO instrument. This abstract presents Phase I results, i.e., qualitative phase. Patient stories were used to develop a conceptual framework covering the key concerns of patients, and to develop a set of preliminary items. Items were grouped into clinically meaningful scales and instructions and four-point response options were developed. The scales were refined by obtaining feedback from a sample of surgical experts and patients. **RESULTS:** From 59 patient interviews, we developed a conceptual framework. Over 3,500 preliminary items were developed and used to inform the following 17 independently functioning scales: 1) appearance scales measuring the body, abdomen, upper arms, buttocks, inner thighs, hip and outer thighs, skin and scars; 2) quality of life scales measuring body image, sexual, psychological and social wellbeing, physical function and symptoms; and 3) process of care scales measuring satisfaction with information, doctor and office staff. **CONCLUSIONS:** Phase II involves a multicentered field-test in Canada and the USA. Rasch Measurement Theory analysis will be used to determine which items to retain in each scale based on their performance against a standard set of psychometric criteria.

PSY53

THE USE OF PREFERENCE BASED MEASURES IN HAEMOPHILIA: IS THE CURRENT EVIDENCE BASE USEFUL FOR EVIDENCE BASED DECISION MAKING?

Tolley K[1], Miners A[2], Brazier J[3], Pericleous LM[4], Sharma T[4], Petersen J[4], Lonergan T[5]
[1]*Tolley Health Economics, Buxton, UK,* [2]*London School of Hygiene and Tropical Medicine, London, UK,* [3]*University of Sheffield, Sheffield, South Yorkshire, UK,* [4]*Novo Nordisk A/S, Soborg, Denmark,* [5]*Tolley Health Economics, Derbyshire, UK*