gain category (3.45, 3.97) and increased with increasing categorical weight loss: B 7.63, 7.61; C 10.61, 10.92; D 12.61, 16.50; E 17.27, 15.35. Changes in SF-36 physical component summary scores, but not mental component summary scores, followed a similar pattern. **CONCLUSIONS:** We observed higher proportions of subjects achieving greater categorical weight loss with liraglutide 3.0 mg versus placebo as adjunct to D&E. With greater categorical weight loss, greater improvements in IWQOL-Lite total and SF-36 physical component summary scores were observed.

PSY92

COGNITIVE TESTING OF A MODIFIED VERSION OF THE FACES PAIN SCALE-REVISED IN CHILDREN WITH SICKLE-CELL DISEASE

Naegeli AN[1], Heath L[2], Turner-Bowker DM[3], Flood E[4], Dampier C[5]
[1]*Eli Lilly, Indianapolis, IN, USA,* [2]*Eli Lilly and Company, Indianapolis, IN, USA,* [3]*Quintiles (work conducted while at ERT, Pittsburgh, PA), Cambridge, MA, USA,* [4]*ICON, Morristown, NJ, USA,* [5]*Emory University School of Medicine, Atlanta, GA, USA*

OBJECTIVES: Sickle-cell disease (SCD) manifests clinically as severe pain episodes in various locations of the body. Patients with SCD also experience chronic daily pain, which profoundly affects quality of life. Cognitive interviews were conducted to evaluate comprehension and usability of a modified version of the Faces Pain Scale-Revised (Modified FPS-R), which asks children to rate their worst pain today using an electronic device. **METHODS:** In-person interviews were conducted in the US with children aged 4-17 years with SCD and their parent/legal guardian. Children who were unable to read or needed guidance were assisted by their parent/legal guardian using written administration guidelines. Children were asked questions about their pain experience, understanding of the instrument, and ability to use the electronic device. Parents/legal guardians were debriefed on the administration guidelines, assisting their child, use of the electronic device, and their child's pain experience. **RESULTS:** The sample included 22 African American children (13 females/9 males; 7=4-5 year-olds, 12=6-11 year-olds and 3=12-17 year-olds). Pain was most commonly reported to occur in the legs, back, arms, stomach, or head. Those aged ≥7 years were able to demonstrate good understanding of the Modified FPS-R item and response scale and ability to use the electronic device. Children 4-6 years were generally not familiar with "discomfort" and many did not know the meaning of "pain". It was unclear whether these children were able to consider their worst pain over the course of the day and respond accordingly. Parents/legal guardians noted that the instrument instructions were clear and that the administration guidelines provided simplified, standardized direction for young children and those unable to read without assistance. **CONCLUSIONS:** The Modified FPS-R used with the administration guidelines where parental assistance is needed, is an appropriate measure of sickle-cell pain over the course of a day for children aged ≥7 years.

PSY93

REPORTING INSTRUMENTS OF PATIENT REPORTED OUTCOMES IN ORPHAN DISEASE

Kaushik P, Bhalla S, Jain A
PAREXEL, Chandigarh, India

OBJECTIVES: To identify patient-reported outcomes (PROs) evaluating quality of life (QoL) in Gaucher Disease (GD) after formation of Rare-Diseases Program in 2010 by the Center for Drug Evaluation and Research under United States Food and Drug Administration with a mission to acknowledge patient's perceptions on treatment benefits. **METHODS:** Embase® and MEDLINE®databases were searched from January 2010 to May 2015. Studies published in English language were included irrespective of study designs by two independent reviewers with discrepancies reconciled by a third independent reviewer. **RESULTS:** Out of 325 studies retrieved from biomedical databases, only nine studies reporting seven different PRO instruments were included. Details of study conduct were adequately reported with no significant difference between treatment groups. Six generic instruments were identified from the included studies, Short Form Health Survey with 36-items (SF-36; 5 studies), 12-items (SF-12; 1 study), the European Quality of Life - Five Dimensions (EQ-5D; 2 studies), Functional Assessment of Chronic Illness Therapy - Fatigue (FACT-F; 1 study), Brief Pain Inventory (BPI; 1 study), and the Visual Analogue Scale (VAS; 1 study). Only one study used the disease-specific Type 1 Gaucher Disease - Disease Severity Scoring System (GD-DS3) providing a reliable method of assessing both intra- and inter-patient severity indicating an impact of pain on the QoL. Overall, the results correlated with clinical outcomes in GD patients demonstrating poorer physical functioning than healthy controls. A review of HTA submissions showed that current evidence on QoL was not sufficient to assess disease impact with the French HTA agency (HAS) particularly encouraging the need to assess QoL to support evidence on treatment effects. **CONCLUSIONS:** Variability in use of PRO instruments was seen among the included studies. Disease specific PRO measures are highly acceptable and need to be developed in orphan disease for better evaluation of QoL.

PSY94

IMPACT OF PAIN SEVERITY ON PATIENT-REPORTED OUTCOMES OF INDIVIDUALS WITH CHRONIC LOWER BACK PAIN IN JAPAN

Montgomery W[1], Vietri J[2], Shi J[3], Kariyasu S[4], Ogawa K[4]
[1]*Eli Lilly Australia, West Ryde, Australia,* [2]*Kantar Health, Horsham, PA, USA,* [3]*Kantar Health, Princeton, NJ, USA,* [4]*Eli Lilly Japan K.K., Kobe, Japan*

OBJECTIVES: To quantify the impact of pain severity on patient-reported outcomes among individuals diagnosed with chronic lower back pain (CLBP) in Japan. **METHODS:** Data came from the 2012 Japan National Health and Wellness Survey (N=29,997), a web-based survey of individuals in Japan aged ≥18 years. This analysis included respondents diagnosed with lower back pain of ≥3 months' duration. Measures included the SF-36v2, Patient Health Questionnaire (PHQ-9), Generalized Anxiety Disorder 7-item (GAD-7) scale, Work Productivity and Activity Impairment questionnaire, and self-reported all-cause healthcare visits (6 months). Generalized linear models with appropriate link functions were used to assess the relationship between outcomes and severity of pain in the prior week as reported on a visual analog scale ranging from 0 (No pain) to 10 (Pain as bad as you can imagine) while controlling for length of diagnosis, sociodemographics, and general health characteristics. **RESULTS:** A total of 346 respondents were included in the analysis; mean age was 55 years, 55% were male, and 55% were employed. Pain severity was 3/10 for the 1st quartile, 5/10 for the median, and 7/10 for the 3rd quartile of this sample. Increasing severity was associated with lower scores for mental (MCS) and physical component summaries (PCS) and SF-6D health utility, higher depression (PHQ-9) and anxiety (GAD-7) scores, greater absenteeism and presenteeism, greater activity impairment, more healthcare provider visits, and more emergency visits (all $p<0.05$). As pain severity increased from the 1st to 3rdquartile, MCS, PCS, and SF-6D all declined more than the minimally important difference, while work and activity impairments approximately doubled. **CONCLUSIONS:** The impact of CLBP on HRQoL, depression and anxiety symptoms, impairment to work and daily activities, and healthcare use increases with the severity of pain. Interventions reducing severity of pain may improve numerous health outcomes even if the pain cannot be eliminated.

PSY95

THE RELATIONSHIP BETWEEN DISEASE SEVERITY AND QUALITY OF LIFE IN PATIENTS WITH MODERATE TO SEVERE PSORIASIS

Geale K[1], Henriksson M[2], Schmitt-Egenolf M[1]
[1]*Umeå University, Umeå, Sweden,* [2]*Linköping University, Linköping, Sweden*

OBJECTIVES: Psoriasis is prevalent in approximately 3% of the population, and often results in significant quality of life losses. The aim of this study was to investigate the relationship between disease severity and quality of life (QOL) measured by the psoriasis area severity index (PASI) and EQ-5D, respectively. **METHODS:** Longitudinal data from a population-based registry in Sweden (PsoReg) were analysed. PsoReg includes patients with moderate to severe psoriasis receiving systemic treatment from a specialist with data available from 2006 to 2014. The analysis was conducted using all complete observations for adult patients at each healthcare contact date. A fixed effects (FE) model was estimated, where time-invariant patient-specific effects were modelled. Time-invariance refers to variables that do not change over time, such as sex or personality. PASI, PASI squared, age, body mass index, smoking status, and presence of psoriatic arthritis were included in the regression as independent variables. PASI squared accounts for a nonlinear relationship between PASI and EQ-5D. The model was tested for the appropriateness of random effects and existence of group-wise homoscedasticity, both of which were rejected at an alpha level of 0.01. Therefore the final model used FE and robust standard errors. **RESULTS:** The estimation utilised 15,099 observations in 3,838 groups, resulting in an average of 3.9 observations per patient. The results indicate that each unit increase in PASI is correlated with a reduction in EQ-5D, but at a decreasing rate: the estimates of PASI and PASI squared were -0.0178 ($p<0.001$) and 0.0002 ($p<0.001$) respectively. This implies that an increase in PASI of 10 (20) units results in a decrease in EQ-5D of 0.1571 (0.2717) holding other variables constant at their respective means. The (adjusted) R2 was (0.5279) 0.6481. **CONCLUSIONS:** QOL in psoriasis patients is decreasing as disease severity increases, but at a decreasing rate.

PSY96

EVALUATION OF PATIENTS' QUALITY OF LIFE OF PREOPERATION AND EARLY POSTOPERATION AFTER TOTAL HIP AND KNEE ARTHROPLASTY

Numanoglu Tekin R[1], Yilmaz F[1], Oksuz E[2], Malhan S[2]
[1]*Baskent University, ankara, Turkey,* [2]*Baskent University, Ankara, Turkey*

OBJECTIVES: Total hip and knee arthroplasty operations are commonly performed to improve patients' quality of life. The aim of this study is evaluation of patients' quality of life of preoperation and early postoperation after total hip and knee arthroplasty. **METHODS:** This study included 26 patients undergoing total hip and knee arthroplasty. After the literature review, 15-items questionnaire was prepared for collecting data related to sociodemographic, medical and functional status information, including age, sex, previous hip and knee arthroplasty and pain level. Quality of life were measured by using the EQ-5D and Visual Analog Scale (VAS). During the study, questionarries were administered to patients 2 times: one week prior to operation and 45 days following operation. **RESULTS:** The average age of participants was 67,12 years (SD: 10,527). Participants were composed of 80,8% females and 19,2% males. Small improvements were reported for pain and functional status after the operations, while large changes were seen in the patients' quality of life. The EQ-5D scores improved significantly preoperatively to postopertively ($p<0,05$) (0,322 vs 0,844). Similarly, there was a significant improvement in VAS scores preoperatively to postoperatively ($p<0,05$) (4,72 vs 7,78). **CONCLUSIONS:** Total hip and knee arthroplasties are well accepted as reliable and suitable surgical procedures to return patients to function and improve the quality of life of patients'.

PSY97

HEALTH-RELATED QUALITY OF LIFE IN PATIENTS WITH TRANSTHYRETIN FAMILIAL AMYLOID POLYNEUROPATHY

Inês M[1], Coelho T[2], Conceição I[3], Ferreira LN[4], Carvalho M[3], Costa J[5]
[1]*Instituto de Medicina Molecular, Lisboa, Portugal,* [2]*Unidade Clinica de Paramiloidose, Hospital de Santo Antonio, Porto, Portugal,* [3]*Centro Hospitalar de Lisboa Norte, Lisbon, Portugal,* [4]*Universidade do Algarve, Faro, Portugal,* [5]*Institute 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) factors for TTR-FAP patients. **METHODS:** HRQoL was measured using the validated EuroQoL five dimensions three levels (EQ-5D-3L) questionnaire being the index score calculated trough the Portuguese scoring algorithm. TTR-FAP symptomatic patients specific data (n = 566) extracted from Transthyretin Amyloidosis Outcomes Survey (THAOS) registry. Demographic variables include gender and age. Clinical variables include disease onset (early/late), polyneuropathy disability (PND) score, liver transplant and pharmacologic treatment. Econometric analyses were carried out to identify factors that impact TTR-FAP HRQoL. **RESULTS:** In a scale from -0.50 to 1.00 the average utility score 0.50(0.37) for symptomatic TTR-FAP patients. Within TTR-FAP population, significant statistical effect (p-value <0.005) was

observed for age, pharmacologic treatment and disease severity. No significant statistical effect was observed for gender, late onset patients (>50 years) and liver transplant. **CONCLUSIONS:** The preference-based utility measures used in this study adequately disentangle TTR-FAP impact on patient's quality of life and allow discriminating across different TTR-FAP clinical severity states, interventions and demographic characteristics. Assuming that these values represent the patients' preferences and the utility associated with their health state, the results presented in this study may be used in future health technologies cost-utility studies.

PSY98

HEALTH-RELATED QUALITY OF LIFE (HRQOL) IN SPLENECTOMIZED IMMUNE THROMBOCYTOPENIA (ITP) PATIENTS – A TARGETED LITERATURE REVIEW

Lebioda A[1], Batscheider A[2]
[1]*Amgen GmbH, Munich, Germany,* [2]*IMS Health, Munich, Germany*

OBJECTIVES: Immune thrombocytopenia (ITP) is associated with a risk of spontaneous and excessive bleeding. Among the treatment options are medical therapies and splenectomy – a major intervention with lifelong consequences for the patient. Both ITP and ITP-treatment may impact health-related quality of life (HrQoL); therefore, a literature search was conducted to assess HrQoL of splenectomized compared to non-splenectomized ITP patients. **METHODS:** A targeted literature search was conducted via Ovid (Medline, Embase, and Cochrane) in September 2014. Search strings were based on PICO-criteria (Patient, Intervention, Comparison and Outcomes). Inclusion criteria were: studies of adult patients with ITP that contained information on HrQoL; clinical trials, RCTs, meta-analyses, observational trials, retrospective studies or systematic reviews; manuscripts and congress abstracts in English or German language. **RESULTS:** The search identified 148 potentially relevant publications. After removing duplicates, 120 titles and abstracts were screened. Following the title/abstract screening, a full text screening (n=21) identified six relevant publications. HrQoL was assessed in these six publications using generic (SF-36, n=2; EQ-5D, n=1) and disease-specifc (ITP-PAQ, n=4) instruments. Two studies using generic instruments reported no difference in HrQoL between splenectomized and non-splenectomized patients. Of the four studies using the ITP-PAQ, two reported no difference and two found worse HrQoL in splenectomized versus non-splenectomized patients. In the only study that included both generic (SF-36, EQ-5D) and ITP-specific measures, results were inconsistent, as they varied in outcomes. Overall detailed results were not always provided and HrQoL was not reported according to response to therapy in any study. **CONCLUSIONS:** The impact of splenectomy on HrQoL in patients with ITP is inconclusive and inconsistently reported in the literature, due to use of different instruments, diverse patient characteristics, and pooling of data among responding and non-responding patients. Further studies are needed to address this question within clearly-defined populations.

PSY99

ASSESSING THE TREATMENT BURDEN FOR GROWTH HORMONE DEFICIENCY (GHD) IN CHILDREN: CONCEPT ELICITATION RESULTS SUPPORTING THE DEVELOPMENT OF THE TREATMENT BURDEN MEASURE FOR CHILDHOOD GHD (TB-CGHD)

Brod M[1], Højbjerre L[2], Wilkinson L[2], Alolga SL[1], Rasmussen MH[2]
[1]*The Brod Group, Mill Valley, CA, USA,* [2]*Novo Nordisk A/S, Søborg, Denmark*

OBJECTIVES: Treatment for children with GHD involves daily injections which may begin at a very early age and continue throughout childhood. However, the treatment burden (TB) for children and their parents has not been examined. The purpose of this study was to support the content validity of a new GHD-specific TB measure, with versions for patient-reported outcome (PRO) for older children, and observer-reported outcome (ObsRO) and PRO for parents/guardians. **METHODS:** Focus groups and interviews were conducted with 39 children (aged 8–12) with GHD, 31 parents of children (aged 4–12) and eight clinical experts in three countries (Germany, UK, US). Interviews were analysed and coded using adapted grounded theory. A conceptual model of TB was developed and items were generated and then cognitively debriefed. **RESULTS:** Qualitative analysis found saturation was reached with three domains for child TB and two domains for parent TB. Child domains (and major proximal subdomains) were: Physical (pain, 33% and bruising, 19%), Interference (Interference with overnight or other activities, 29% and Time needed emotionally to prepare for treatment, 23%) and Emotional (Worry, about injections, 37%, Unhappiness with injection frequency, 25%, and Fear, 22%). Parent TB domains were Emotional (Worry or anxiety about treatment or treatment administration, 58% and Worry about causing pain, 42%) and Interference (Time spent preparing and administering injection, 42%, Interference with travel/vacation, 42%, and Interference with daily/family routines, 35%). The items were cognitively debriefed in a new sample (N=26: 13 children, 13 parents) and, based on findings, it was determined that the PRO version was appropriate for children aged 9–12. The final measure includes 17 items (child PRO and parent ObsRO versions) with a 12-item parent PRO module. **CONCLUSIONS:** The conceptual validity of the TB-CGHD is supported by these qualitative findings and the measure is now ready for psychometric validation.

SYSTEMIC DISORDERS/CONDITIONS – Health Care Use & Policy Studies

PSY100

TOWARDS CREATING A NATIONAL SYSTEM OF CARE FOR PATIENTS WITH ORPHAN DISEASES

Rubtsova I, Shilkina O, Lishchyshyna O
State Expert Centre of the Ministry of Health of Ukraine, Kyiv, Ukraine

OBJECTIVES: There is no integrated approach to the problem of orphan diseases (OD) in Ukraine. Diagnosis of these illnesses is often complicated and delayed, the treatment is often ineffective because of the lack and unavailability of essential medicines and methods of treatment, the quality of life for most patients remains poor. The Law of Ukraine 'On amendments to the Basic Laws of Ukraine on Health Care on prevention and treatment of rare (orphan) diseases' has been recently issued. It governs the measures of OD prophylaxis. **METHODS:** According to the Law, in order to improve OD patient care, a work on medical and technological documents on such topics as mucopolysaccharidosis, Gaucher disease, phenylketonuria, hemophilia was launched. The development of medico-technological documents was done in a few steps. Firstly, the search for clinical guidelines was conducted in the GIN library and other sources. Secondly, the primary selection and appraisal with AGREE II instrument have been carried out. The next step was adaptation of clinical guidelines and development of clinical protocols of the medical care. It was conducted by multidisciplinary working groups including leading specialists in clinical genetics, health care managers and patients organizations. **RESULTS:** Today the clinical protocols of treatment of mucopolysaccharidosis patients, based on evidence of the effectiveness of medical technologies, pharmacotherapy and organizational principles of its provision, were approved. The documents contained modern approaches for diagnostics and treatment of mucopolysaccharidosis, which are ajusted to the health care system of Ukraine. **CONCLUSIONS:** Harmonization of treatment practices of OD in Ukraine with international best practice will bring the treatment of these states in Ukraine to a new level and provide thorough measures aimed at the development of screening and prevention programs, diagnostics and treatment, as well network collaboration among health services, educational institutions, public etc.

PSY101

MEDICAL CARE AND COSTS OF PATIENTS WITH SYSTEMIC LUPUS ERYTHEMATOSUS IN TAIWAN

Cheng J[1], Huang C[2], Hsu C[2]
[1]*Chang Gung University, Tao-Yuan, Taiwan,* [2]*National Health Research Institutes, Miaoli, Taiwan*

OBJECTIVES: Systemic lupus erythematosus (SLE) is a chronic autoimmune disorder that can cause multi-organ damage, leading to reduced life expectancy, lower quality of life, and high medical expenditures. Therefore, long-term and comprehensive disease management and monitoring are important. This study aimed to examine treatment pattern, quality of SLE care, and utilization and costs of medical care of SLE patients. **METHODS:** The National Health Insurance Research Database was adopted to identify patients diagnosed with SLE before 2010 through their eligibility for catastrophic illness of SLE, and examine their use and costs of health services in 2010. Logistic regression models and generalized linear models were adopted for analyses. **RESULTS:** The majority of 744 patients with SLE was female (85%), older than 40 years old (54%), and having been diagnosed with SLE for more than five years (68%). Sixty-one percent of them received corticosteroids, 59% received antimalarials, and 36% received immunosupressants. Annual tests for complete blood count, creatinine, and serum levels of complement component C3/C4 and anti-dsDNA antibodies were received by 85%, 78%, and 76% of the subjects, respectively. Forty-four percent of them received annual evaluation of cardiovascular risk, and 3% of them received influenza vaccination. Average number of outpatient visit was 33.47, and SLL-related visit was 12.82. Twenty-two percent of them were admitted to hospital during the year, and the average number of hospital admission was 0.48. The average annual medical cost was NTD105,059, and the average SLE-related medical cost was NTD29,770. Shorter time since SLE diagnosis was associated with more intense treatment, better adherence to recommendations for annual tests, and higher SLE-related costs. **CONCLUSIONS:** This study identified treatment pattern, quality of SLE care, and use and costs of patients with SLE. Disease management and monitoring need to be improved.

PSY102

THE RHEUMATOLOGIST'S PERSPECTIVE IN DIAGNOSTIC COURSE AND MANAGEMENT OF FAMILIAL MEDITERRANEAN FEVER

Karadag O[1], Kilic L[2], Erden A[1], Kasifoglu T[3], Karaaslan Y[4], Kiraz S[1], Dokuyucu O[5]
[1]*Hacettepe University, Ankara, Turkey,* [2]*Yenimahalle Research Hospital, Ankara, Turkey,* [3]*Eskisehir Osmangazi University Medical School, Eskisehir, Turkey,* [4]*Ankara Numune Research Hospital, Ankara, Turkey,* [5]*Novartis, Istanbul, Turkey*

OBJECTIVES: To investigate the global perspectives of Turkish Rheumatologists regarding expected FMF prevalence, undiagnosed patients ratio, arguments in diagnostic procedure and management of FMF. **METHODS:** A questionnaire consist of estimated frequency, diagnostic modalities and follow up characteristics in addition to therapeutic approach was set up. Even though a regular colchicine use, ≥1 attack / month during 6 months was accepted as colchicine resistant. All members of Turkey Society for Rheumatology from country wide (n=235) were invited to fill in the web-based questionnaire for FMF by e-mail. Totally 108 members (45.9%) answered the questionnaire. **RESULTS:** Estimated prevalence was median 0.2% (IQR25-75: 0.1-0.7) for FMF. Only 51.4±19.0 % of these patients had been diagnosed according to Rheumatologists opinion. Time to diagnosis from the first symptom was more than 3 years in 49.5% of patients. Description of typical attacks in anamnesis is the most frequent parameter in diagnosis of patients whereas in 26.7 %of patients MEFV mutations were required for diagnosis. 53.5 ±21.3 % of patients were on regular follow up and 75.7% of these follow up was with a 3-6 months period. Frequency of Colchicine resistant patients was expected in 7.2%. The use of biologic agents in the patients followed up by these physicians was expected as 3.4%. **CONCLUSIONS:** Since half of the estimated prevalence was expected as undiagnosed and patients have a 3-years delay in diagnosis, Medical and Social Activities are required to increase the awareness of FMF. Less than 5 percent of patients required biologic agents in FMF.

PSY103

IMPACT OF BLOOD TRANSFUSIONS ON HOSPITAL LENGTH OF STAY AND MORTALITY: A SINGLE-CENTER EXPERIENCE

Félix J[1], Alcobia A[2], Soares A[2], Bastos A[2], Amaro A[2], Oliveira C[2], Rabiais S[1], Afonso-Silva M[1], Andreozzi V[1], Vandewalle B[1], Ferreira D[1]
[1]*Exigo Consultores, Alhos Vedros, Portugal,* [2]*Pharmacy, Hospital Garcia de Orta, E.P.E., Almada, Portugal*