pain or bleeding completed a pain history and five PRO instruments (EQ-5D-5L, Brief Pain Inventory v2 [BPI], SF-36v2, International Physical Activity Questionnaire [IPAQ], and Hemophilia Activities List [HAL]) during routine clinic visits. To assess concordance of individual questionnaire items and correlation of domain/global scores using intraclass correlation coefficient (ICC), initial patients (target 125-150) were approached to complete the PROs at the end of their 3-4 hour visit in a similar non-bleeding state. **RESULTS:** From October 2013-October 2014, 381 patients enrolled; 164 (88% of initial 187) completed the retest. Median age of retest cohort was 33.9 years (Q1, Q3; 26.9, 46.0). Median time for completion of the initial survey with five PROs was 36.0 minutes and for the retest was 21.0 minutes. Median/mean time between tests was 1.5/1.6 hours. The majority of subjects had hemophilia A (74.4%) and were white-non-Hispanic (72.6%); 48.7% were married, 62.6% had some college or graduate-level education, 80.7% were employed, and 61.0% were overweight or obese. HCV was more common than HIV (49.4% vs 16.5%); 61.0% self-reported arthritis/bone/joint problems. Median/mean test-retest concordance was: EQ-5D-5L, 80.0%/79.1%; BPI, 54.5%/58.9%; SF-36v2, 77.8%/76.4%; IPAQ, 100%/100%; and HAL, 77.4%/75.9%. ICC for test-retest reliability were: EQ-5D-5L Health Index, 0.890; BPI–severity, 0.950; BPI–interference, 0.920; IPAQ total activity, 0.940; SF-36v2 overall health, 0.910; HAL total score, 0.970. **CONCLUSIONS:** All five PROs are reliable in adult PWH. Therefore, the choice of instrument to be used for research or clinical care should be driven by instrument characteristics other than reliability.

PSY59

MEASUREMENT PROPERTIES OF WEB BASED LUPUSPRO, A DISEASE TARGETED OTCOME TOOL, AMONG ITALIAN PATIENTS WITH LUPUS

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OBJECTIVES: Patient reported outcomes provide important information on the comprehensive impact of Systemic Lupus Erythematosus (SLE) on daily lives of patients. It also fosters patient physician communication, patient engagement and satisfaction with medical care. LupusPRO is a disease targeted patient reported outcome tool that has good measurement properties (Jolly et al., 2012) and has been validated in several countries. Herein we report the measurement properties of the Italian translation of the LupusPRO that was administered using a web based format. **METHODS:** LupusPRO was translated using forwards and back translation method into Italian language. It was pretested among 5 native Italian speaking subjects. As part of another study, 344 patients with SLE diagnoses were approached through the Patients organization network, asked to provide their demographics, medications and responses to the online LupusPRO. We evaluated internal consistency reliability (ICR) of the LupusPRO domain items, floor-ceiling effects, known groups validity (KGV) and confirmatory factor analysis (CFA). ICR was tested using cronbach's α. KGV was tested against current use of corticosteroids, with the hypothesis that SLE patients currently on corticosteroids would have worse health status than without. Goodness of fit was evaluated in the CFA. **RESULTS:** Mean (SD) age and duration of disease were 39.7 (11.1) and 11.7 (9.3) yrs. Ninety one percent were women. Sixty four percent were on corticosteroids and sixty six percent were on hydroxychloroquine at the time of the study. ICR and Floor-ceiling effects were acceptable. The hypothesis about corticosteroids was supported for many subscales. On CFA, with few exceptions the items correlated highly (r>0.5) with their hypothesized scale. The CFI was 0.938 and TLI was 0.973 (>0.9 is considered good model fit). The RMSEA was 0.093 (<0.1 is considered acceptable model fit). **CONCLUSIONS:** Web version of LupusPRO Italian translation shows fair psychometric properties in Systemic Lupus Erythematosus patients.

PSY60

IMPROVEMENT IN HEALTH-RELATED QUALITY OF LIFE FOR SICKLE CELL DISEASE PATIENTS TREATED WITH CHEMOTHERAPY-FREE ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION: A PILOT STUDY

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OBJECTIVES: Allogeneic hematopoietic stem cell transplantation (HSCT) can cure Sickle Cell Disease (SCD) but traditional chemotherapy-based conditioning regimens for HSCT are associated with substantial morbidity and mortality. The objective of this study was to determine if an innovative approach of chemotherapy-free, non-myeloablative HSCT with alemtuzumab is associated with improved health-related quality of life (HRQoL) in adult SCD patients. **METHODS:** Thirteen high-risk SCD patients underwent chemotherapy-free HSCT at a large urban academic medical center using human leukocyte antigen (HLA)-matched, related donors. Indications for transplantation in this cohort included frequent pain crises, stroke, and/or ≥2 lifetime episodes of acute chest syndrome. Generic HRQoL was assessed using the SF-36 in 9 patients who were assessed pre-HSCT and at 365 post-HSCT. Magnitude of effect was evaluated for SF-36 norm-based subscales and the SF-6D summary scores based on standardized response mean (SRM) (SRM>0.5=medium; SRM>0.8=large effect). **RESULTS:** Of 13 patients, all survived and 9 had completed 365 day post-HSCT assessments by time of analysis. Mean change scores 365-day post-HSCT were 4.6 (SD 21.3) for physical functioning (PF) (SRMPF=0.2), 9.0 (SD 23.0) for role physical (RP) (SRMRP=0.4), 17.1 (SD 14.2) for bodily pain (BP) (SRMBP=1.20), 18.6 (SD 16.0) for general health (GH) (SRMGH=1.16), 15.2 (SD 10.5) for vitality (VT) (SRMVT=1.45), 13.0 (SD 17.2) for social function (SF) (SRMSF=0.76), 5.1 (SD 14.7) for role emotional (RE) (SRMRE=0.34), 5.7 (SD 9.8) for mental health (MH) (SRMMH=0.59) and 0.21 (SD 0.13) forSF-6D index score (SRMSF6D=1.66). **CONCLUSIONS:** Our pilot study demonstrated a large magnitude of improvement in HRQoL in terms of BP, GH, VT and overall health according to the SF-6D for SCD patients undergoing chemotherapy-free, non-myeloablative HSCT with alemtuzumab. These promising results, which are the first report of the impact of this innovative treatment on HRQoL in adults, warrant further investigation with more patients under treatment and longer-term follow-up.

SYSTEMIC DISORDERS/CONDITIONS – Health Care Use & Policy Studies

PSY61

CHARACTERISTICS OF PATIENTS WITH SYSTEMIC LUPUS ERYTHEMATOSUS (SLE) CURRENTLY EXPERIENCING FLARES IN CLINICAL PRACTICE SETTINGS IN EUROPE (EU)

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OBJECTIVES: To assess the characteristics of SLE patients currently experiencing flares in Europe. **METHODS:** A multi-center retrospective medical chart review of adult (16-89 yrs) SLE patients was conducted in 1Q2014 among rheumatologists/internal medicine physicians in UK/France/Germany/Italy/Spain (5EU). Physicians were recruited from a geographically representative sample in each country. Approx. 5 consecutive eligible persistent active or relapse remitting SLE patients currently managed as part of usual care were identified within the study observation window. Physicians abstracted de-identified patient data on disease characteristics, lab values and treatment patterns. Patient disease status and humanistic burden was assessed by physician per clinical judgment & patient interaction. Patients experiencing a flare were identified for analysis. **RESULTS:** 135 SLE patients who were experiencing flares were included in the analysis (UK:45/France:22/Germany:20/Italy:20/Spain:28). Mean age(yrs) was 5EU:40.6/UK:44.4/France:39.3/Germany:36.9/Italy:41.0/Spain:37.9; % female was 5EU:78%/UK:87%/France:59%/Germany:75%/Italy:70%/Spain:86%; % full-time employment was 5EU:33%/UK:20%/France:32%/Germany:30%/Italy:50%/Spain:43%; % part-time employment was 5EU:18%/UK:29%/France:9%/Germany:10%/Italy:10%/Spain:18%; % on sick leave was 5EU:17%/UK:9%/France:23%/Germany:25%/Italy:15%/Spain:21%. Top-5 organ manifestations observed were musculoskeletal (5EU:88%/UK:93%/France:100%/Germany:65%/Italy:95%/Spain:82%), mucocutaneous (5EU:85%/UK:96%/France:91%/Germany:65%/Italy:80%/Spain:82%), haematologic (5EU:56%/UK:56%/France:41%/Germany:60%/Italy:50%/Spain:68%), renal (5EU:33%/UK:33%/France:23%/Germany:50%/Italy:45%/Spain:21%) and pulmonary (5EU:27%/UK:38%/France:5%/Germany:10%/Italy:35%/Spain:32%). Percentage of patients with low C3 (5EU:73%/UK:55%/France:62%/Germany:85%/Italy:90%/Spain:89%), low C4 (5EU:78%/UK:59%/France:90%/Germany:90%/Italy:75%/Spain:89%) and positive anti-ds-DNA (5EU:90%/UK:80%/France:100%/Germany:95%/Italy:95%/Spain:93%) were high. Among those with lab measures, mean ESR (mm/h) was 5EU:50.1/UK:44.4/France:47.6/Germany:41.2/Italy:54.6/Spain:63.8; mean hemoglobin (g/L) was 5EU:10.4/UK:9.8/France:11.1/Germany:10.5/Italy:10.5/Spain:10.7. Humanistic burden (reported via physician ratings, on a scale of 1 (most impact) to 7 (least impact)) was (mean scores): patient ability to perform every-day tasks - 5EU:4.4/UK:4.4/France:5.1/Germany:3.8/Italy:4.5/Spain:4.0; patient ability to interact fully with family and friends - 5EU:4.9/UK:4.9/France:5.5/Germany:4.2/Italy:5.0/Spain:4.9; patient ability to work/keep employment - 5EU:3.9/UK:4.2/France:4.2/Germany:2.8/Italy:4.2/Spain:3.7. Percentage of patients hospitalized >=1 in the past-year was 5EU:56%/UK:40%/France:68%/Germany:65%/Italy:65%/Spain:57%. **CONCLUSIONS:** SLE patients experiencing flares had significant clinical and humanistic burden across the studied countries. Further scrutiny is warranted to assess the modalities of care delivered to this patient group to alleviate their burden.

PSY62

TRENDS IN APPROVALS OF NEW DRUGS WITH ORPHAN DESIGNATION IN THE US (1983-2014)

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OBJECTIVES: Orphan drugs are indicated for rare diseases and conditions. We assessed trends in approvals of new drugs with orphan indications in the US and in the prevalence of orphan drugs approved by the FDA from 1983 to 2014 compared to non-orphan drug approvals in the same time frame **METHODS:** The study used regulatory and prevalence data derived from the US FDA, the European Medicines Agency (EMA), and other publicly available sources. Descriptive statistics and the chi-square test were performed in the study. **RESULTS:** A total of 103 BLAs and 799 NMEs were approved by the FDA from 1983 to 2014. The percentage of new drugs with orphan designation at approval was 43.7% for BLAs and 20.7% for NMEs (p<0.001). Orphan drugs represented 13.9% of the new drugs approved from 1983 to 1989, 21.2% in the 1990s, 25.5% in the 2000s, and 33.8% from 2010 to 2014. Information about the prevalence of disease was available for 73.7% of the orphan drugs. Indications approved for use in diseases with a prevalence of less than 1000 patients (i.e.: ultra-orphan drugs) represented 8.4% of all new orphan drugs. Ultra-orphan drugs represented 12.5%, 3.6%, 7.7%, and 15.8% of all orphan indications approved in the US from 1983-1989, 1990s, 2000s, and 2010-2014, respectively. **CONCLUSIONS:** This study found an increase in the percentage of drugs approved by the FDA for orphan diseases and conditions. Ultra orphan drugs revealed a greater increase over time than other orphan drugs as a proportion of all new drugs approved by the FDA. The limited innovation in diseases with large prevalence, the incentives provided by the Orphan Drug Act of 1982 and favorable reimbursement for orphan drugs in the US health care system may explain the increase in the approval of orphan drugs in the US.

PSY63

DETERMINING PATTERNS OF OPIOID MISUSE AND MISPRESCRIBING IN SOUTH CAROLINA

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