

EORTC QLQ-C30 cancer mapping algorithms ranged from 0.70 to 0.92. Utility values derived from the two versions of the MF-8D were also within this range, at 0.79 (equal weighting) and 0.73 (Rasch weighting). Clinical opinion indicated that the MF-8D captures the key symptoms of PV (e.g., pruritus, abdominal pain/discomfort, bone pain, night sweats) better than the other cancer algorithms. **CONCLUSIONS:** The MF-8D may be more appropriate than other cancer algorithms for estimating utility values for PV patients, as it includes the most important symptoms of PV. This measure is expected to better capture treatment-specific differences affecting HRQoL that are important for the economic evaluation of emerging therapies.

PSY48

THE IMPACT OF INCREASING SEVERITY OF HEREDITARY HAEMOCHROMATOSIS ON HEALTH STATE UTILITY VALUES

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OBJECTIVES: Hereditary haemochromatosis (HH) is a common autosomal recessive disorder amongst persons of European heritage. Elevated hepcidin production increases the absorption of dietary iron, which is stored in the parenchymal tissues of the heart, liver and pancreas. Treatment consists of regular venepuncture, and if implemented early, prevents organ damage. If untreated, iron overload can be a cause of morbidity and mortality. To date, a lack of robust health economic evidence has been cited as one of the barriers to establishing screening programs for HH. Previous analyses have used unvalidated estimates of health state utility values (HSUV). This is in part due to no published estimates of HSUV. This study sought to estimate HSUV directly from people with different levels of severity of HH of in Australia. **METHODS:** Volunteers with HH were recruited to complete a web-based survey as part of a national cost of illness study for HH. HSUV was estimated using the Assessment of Quality of Life 4D (AQOL-4D) instrument. Severity of HH was categorised according to the method recommended by the European Association of the Study of the Liver. Multivariate regression analysis was performed to identify parameters associated with HSUV. **RESULTS:** Between November 2013 and November 2014, 221 people completed the survey. Increasing severity of HH was negatively associated with HSUV. Mean (standard deviation) HSUV were 0.76 (0.21), 0.81 (0.18), 0.60 (0.27), and 0.50 (0.27) for grades 1–4 HH respectively. **CONCLUSIONS:** Increasing severity of HH is associated with decreasing HSUV. Previous cost utility analyses have used higher HSUV which likely resulted in underestimates of the cost effectiveness of screening programs for HH. The HSUV reported in this paper are the most robust available and their use would improve the validity of future economic models for HH.

PSY49

IMPROVED HEALTH STATUS OF HAEMOPHILIA B PATIENTS TREATED WITH NONACOG BETA PEGOL, A NEW GLYCOPEGYLATED RECOMBINANT FIX PRODUCT WITH PROLONGED HALF-LIFE

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OBJECTIVES: To assess the health status of haemophilia B patients treated with nonacog beta pegol, a novel recombinant Factor IX (FIX) product with prolonged half-life and less frequent dosing requirements. **METHODS:** Severe/moderate haemophilia B (FIX activity level $\leq 2\%$) is an inherited bleeding disorder characterised by recurrent, often spontaneous bleeding episodes, in particular musculoskeletal bleeds. Modern management includes self-infusion of FIX 2–3 times/week to prevent bleeds (prophylaxis) or on-demand treatment (OnD). Haemophilia B patients aged 13–70 years were included in a single-blind phase 3 trial evaluating safety and efficacy of nonacog beta pegol. Patients were treated OnD for 28 weeks, or randomised to once weekly prophylaxis with 10 or 40 IU/kg for 52 weeks. All patients completed the 3 level EuroQoL-5 dimensions (EQ-5D-3L) at baseline and end-of-trial. EQ-5D VAS ranges from 0–100. Higher score represents better health status. Changes in scores were compared to no change using the Wilcoxon signed rank test. **RESULTS:** 74 patients were included in the Full Analysis Set (15 OnD; 30 on prophylaxis with 10 IU/kg dose and 29 on prophylaxis with 40 IU/kg). 56 patients were 18–70 years and 18 were 13–17 years. A significant improvement in EQ-5D index score was observed in the overall set (mean change = 0.04, $p=0.016$), but no significant change was observed for the different dose groups. In subset analysis of the high dose prophylaxis arm, mean EQ-5D VAS was 74.3 \pm 18.0 at baseline and 83.9 \pm 14.5 at end-of-trial, leading to a statistically significant improved mean change in score ($p=0.030$). The change was non-significant in the OnD and low dose prophylaxis arms. **CONCLUSIONS:** In a phase 3 clinical trial, haemophilia B patients treated with the long-acting FIX nonacog beta pegol showed a significant improvement in their health status. Patients treated with the prophylaxis dose of 40 IU/kg/weekly demonstrated significant improvement in health status according to the EQ-5D VAS.

PSY50

USE OF INFLIXIMAB WITHIN A PATIENT SUPPORT PROGRAM: POSITIVE PERCEPTION OF IV INFUSIONS FROM PATIENTS' PERSPECTIVE

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OBJECTIVES: Patients with auto-immune diseases who are treated with infliximab (IFX) intravenous (IV) infusions are managed through a manufacturer-sponsored patient support program (PSP). Previous studies have had conflicting conclusions regarding patient preferences of modes of administration of biologics, with implications of negative perceptions of IV infusions. The aim of this study was to assess patients' experience of IFX IV therapy administered in the PSP and to determine predictors of a positive perception of IV infusions. **METHODS:** In this nationwide, cross-sectional survey, patients currently receiving IFX within the PSP were given

brochures to access a web-based survey (May 5–July 18, 2014) of demographic & disease characteristics, respondents' lifestyle & health ratings, and their perception of IV infusions before and after initiating therapy. The analysis was exploratory and descriptive; data collected was a self-reported ordinal (scale, low-high, 1–10) with medians (IQR) reported. A stepwise (backward) multinomial logistic regression was conducted to determine predictors of a positive perception of IV infusions. **RESULTS:** There were 1,762 respondents from 10,000 distributed brochures (18%). The majority of patients were: bio-naïve, employed, busy/active, and treated for inflammatory bowel disease. Median current health rating was high 8(6–9). The perception of IV infusions improved from initial treatment; majority of patients rated it as 5/10 prior to starting therapy vs. 8/10 after multiple infusions ($p<0.0001$). Predictors of a positive IV experience were 'satisfaction with the PSP coordinator' (OR= 3.0, 95% CI= 1.5–5.7) and 'receiving an accurate description of treatment through the PSP from a physician' (OR=3.6, 95%CI= 2.2–5.9). **CONCLUSIONS:** Although there is selection bias, we found that patients have a positive experience with the PSP and report significant improvements in their perception of IV infusion. Regression analysis suggests that providing patients with an accurate description of the treatment at the PSP can improve the perception of the IV experience.

PSY51

POTENTIAL PATIENT-REPORTED PREDICTORS OF OPENNESS TO AND PREFERENCE FOR BIOLOGIC THERAPY AMONG PATIENTS WITH INFLAMMATORY BOWEL DISEASE

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OBJECTIVES: Intravenous infusions (IV) vs. subcutaneous injection (SQ) biologic products offer advantages and disadvantages to patients with inflammatory bowel disease (IBD). This study examines potential predictors of IV vs. SQ openness/preference among patients with IBD. **METHODS:** Patients (n=263) completed a self-administered, web-based questionnaire assessing demographics, health, disease, treatment characteristics, behaviors, and attitudes. Patients included: U.S. adults (aged ≥ 18) diagnosed with ulcerative colitis (UC; n=140), Crohn's disease (CD; n=123), receiving immunomodulator treatment, but biologics-naïve. Chi square and t tests compared patients open vs. not open or preferring vs. not preferring IV. Logistic regression models predicted openness and preference as a function of potential predictors. **RESULTS:** Among all patients (mean age=45.8, 57.1% female, 23.5% minority, and mean years since diagnosis=10.7), 74.2% were open to starting biologics after discussion with their physicians, 64.6% (n=170) were open to IV, and among those, 34.7% (n=59) preferred IV vs. SQ. Potential predictors of openness to IV included: considering one's physician's advice to be very-to-extremely influential for treatment decisions (OR=4.67; 1.87–11.64), male vs. female (OR=2.94; 1.41–6.10), number of emergency room, hospital, and/or physician visits for UC/CD in the past 6 months (OR=1.24; 1.07–1.43), ever experiencing fever as UC/CD sign/symptom (OR=2.81; 1.21–6.52), and awareness of infusion for UC/CD (OR=2.38; 1.10–5.16); intention to delay biologics treatment due to safety perceptions inhibited openness to IV (odds ratio [OR]=0.48; 95% confidence interval [CI]: 0.35–0.66); all $p<.03$. Potential predictors of preference for IV over SQ included: higher relative openness to office/hospital/medical facility vs. home site of care (OR=5.72: 3.51–9.31), receiving/ever having received infusion (OR=4.11; 1.31–12.95), and detailed discussion of redness/swelling after injection (OR=4.15; 1.29–13.39), all $p<.02$. **CONCLUSIONS:** The current study helps inform patient characteristics, attitudes, and disease and treatment history indicating interest in IV biologics treatment, which can help enable shared decisions to best benefit patients with IBD.

PSY52

IBRUTINIB FOR THE TREATMENT OF MANTLE CELL LYMPHOMA (MCL): EVALUATING THE CORRELATION BETWEEN PATIENT-REPORTED OUTCOMES AND DURABILITY OF RESPONSE IN A PHASE 2 STUDY

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OBJECTIVES: To investigate the relationship between patient-reported outcomes and durability of response using data from a phase 2 study of single-agent ibrutinib for patients with previously-treated MCL. **METHODS:** The phase 2 SPARK study evaluated the efficacy and safety of single-agent ibrutinib in patients with MCL who had received a rituximab-containing regimen and had progressed after ≥ 2 cycles of bortezomib therapy. In this multicenter, single-arm study, patients were enrolled to receive 560 mg/day ibrutinib continuously until progressive disease (PD) or unacceptable toxicity. The primary end point was the overall response rate in response-evaluable patients, as assessed by an Independent Review Committee. Secondary end points included patient-reported outcomes (FACT-Lym). **RESULTS:** Overall, 25 of 110 evaluable patients (22.7%) were considered to have primary resistant disease (PD at first disease evaluation), with 85 of 110 (77.3%) considered as "responders": 22 patients (20%) were considered to have moderate response (stable disease [SD] or better, but with PD within 12 months; MR group), and 63 patients (57.3%) were considered to have durable response (SD or better, maintained for >12 months; DR group). Median progression-free survival for the total evaluable population was 10.5 months; 4.1 months for the MR group and 16.8 months for the DR group. Additionally, 67 of 109 response-evaluable patients who had PRO results (61.5%) achieved a clinically-meaningful improvement in lymphoma symptoms based on