

sensitivity analyses indicates that for these comparisons, based on a threshold of £20,000, Movantik has a respective probability of 91%, 83% and 100% of being cost effective. In a population of LIR patients who are taking step 3 opioids, Movantik is dominant vs. Targin over a 5 year time horizon. **CONCLUSIONS:** Movantik is a cost-effective treatment option for patients with OIC who have experienced inadequate response to laxative(s).

PSY37

COST-EFFECTIVENESS ANALYSIS OF EX-VIVO EXPANDED AUTOLOGOUS CORNEAL EPITHELIAL CELLS CONTAINING STEM CELLS TO REPAIR THE DAMAGED OCULAR SURFACE IN PATIENTS WITH MODERATE TO SEVERE LIMBAL STEM CELL DEFICIENCY DUE TO OCULAR BURNS IN THE UK
Fordham R¹, Ciminata G¹, Madoni A², Magni T², Ardigò D², Pelosi D², Withe M², Sarnelli V³, Deltetto I³, Domini B³

¹University of East Anglia, Norwich, UK, ²Chiesi Farmaceutici Spa, Parma, Italy, ³Ernst & Young, Milano, Italy

OBJECTIVES: Limbal Stem Cell Deficiency (LSCD) is a rare condition characterized by the shortage of limbal stem cells in the eye resulting in corneal conjunctivalization, corneal opacity, visual impairment and even blindness. Recently, the first advanced therapy medicinal product (ATMP) containing stem cells (GPLSCD01) has been recommended for approval by EMA in moderate-severe LSCD due to chemical or physical burn. A Cost Effectiveness Analysis (CEA) was performed, from a public payer perspective, to compare GPLSCD01 in LSCD with conservative treatment, given that, currently, no other medicinal product is approved for this disease. **METHODS:** We analyzed visual acuity and symptoms from 99 patients (average age 46.8 yrs.) treated with GPLSCD01; data were taken from a retrospective, case-series, non-randomized, non-controlled, multicenter clinical study (HLSTM01), covering up to 10 years follow-up. LSCD-impaired visual acuity and symptoms such as pain, burning and photophobia were used in the model to assess the QoL associated with the condition, and Quality Adjusted Life Years (QALY) to compare the outcomes of GPLSCD01 treatment versus conservative management, in a similar patient pool. **RESULTS:** Patients under conservative treatment had between 10.29 and 17.24 QALYs, depending on LSCD severity, whereas patients treated with GPLSCD01 showed between 15.93 and 22.49 QALYs, with a total utility gain between 5.25 and 6.04 QALYs in the GPLSCD01 group, this result being already discounted by 3.0%, in compliance with National Institute for Health Care Excellence (NICE) guidelines. Due to the utility gain, GPLSCD01 would meet NICE conventional ICER thresholds (20,000–30,000 GBP/QALY) up to a treatment cost of 150,000 GBP. **CONCLUSIONS:** GPLSCD01 is a regenerative medicine, offering long-term, potentially life-long effectiveness after single administration. This CEA shows that GPLSCD01 in moderate-severe LSCD provides a significant gain in QALYs compared to conventional, conservative management of the condition, balancing, from a payer perspective, initial higher acquisition costs.

PSY38

COST-EFFECTIVENESS OF HOME-BASED FATIGUE SELF-MANAGEMENT INTERVENTION: A RANDOMIZED CONTROLLED TRIAL
Meng H¹, Friedberg F²

¹University of South Florida, Tampa, FL, USA, ²Stony Brook University, Stony Brook, NY, USA

OBJECTIVES: The cost of lost productivity associated with chronic fatigue has been estimated to be more than £75 million per year in the U.K. Cognitive behavioral Therapy (CBT) based fatigue self-management (FSM) has been shown to be effective in reducing fatigue symptoms. This study aimed to examine the cost-effectiveness of a home-based CBT-FSM intervention as compared to usual care. **METHODS:** 137 Primary care patients between 18 and 65 years of aging who had at least six months of persistent fatigue were randomized into either home-based CBT-FSM intervention (n=89, 45 with actigraph and web diaries and 44 with pedometer and paper diaries) or usual care (n=48). All patients were followed for 12 months post-treatment. Effectiveness was measured by the Fatigue Severity Scale (FSS) and health care resource use was measured by a utilization diary. Resource use categories included provider visits, diagnostic tests, prescription medication use, and use of emergency room, hospital, informal care, and days of lost work. We used ordinary least squares model and generalized linear model to estimate the treatment effect on effectiveness and health care expenditures, respectively. Cost-effectiveness was measured by the incremental cost-effectiveness ratio (ICER) and net monetary benefit. **RESULTS:** The sample had a mean age of 48.5 years and most patients were female (88.3%). Baseline characteristics were similar between the intervention and control groups. Adjusting for baseline characteristics, the intervention had a small non-significant effect on FSS (-0.16, p=0.32) and total expenditures (0.09, p=0.32), resulting an ICER of 388 (bootstrapped 95% CI: -16463, 17239). Cost-effectiveness acceptability curve showed that CBT-FSM had an 85% probability of being cost-effective if societal willingness-to-pay for each 1 point reduction on the FSS. **CONCLUSIONS:** The intervention did not appear to be cost-effective as compared to usual care. The home-based intervention needs to be modified to maintain effectiveness achieved in prior interventions delivered by training professionals.

PSY39

COST-EFFECTIVENESS OF RITUXIMAB VERSUS CALCINEURIN INHIBITORS FOR REFRACTORY MYASTHENIA GRAVIS
Georgieva M¹, Wheeler S¹, Howard Jr JF²

¹Gillings School of Global Public Health, University of North Carolina at Chapel Hill, Chapel Hill, NC, USA, ²University of North Carolina at Chapel Hill, Chapel Hill, NC, USA

OBJECTIVES: Myasthenia gravis (MG) is a rare autoimmune disorder characterized by exacerbations and remissions. The costs of managing MG are higher than those of many other chronic neurological diseases and there are no studies evaluating the cost-effectiveness of treatment alternatives for refractory MG in patients who failed to respond to at least two successive immunosuppressive drugs. This study aimed to assess the incremental costs and benefits of rituximab, cyclosporine, and tacrolimus in the treatment of refractory MG. **METHODS:** We used a Markov

model from the perspective of a US private insurance payer to evaluate the cost-effectiveness of rituximab, tacrolimus, and cyclosporine in a hypothetical cohort of 1,000 patients with refractory MG, aged 20 years and above. We obtained disease transition probabilities, costs and outcomes data from the published literature. We calculated the incremental cost-effectiveness ratios (ICERs) as cost per quality-adjusted life-year (QALY) gained and cost per myasthenic crisis averted after the first two years of treatment and over a patient's lifetime. **RESULTS:** In the first two years after treatment rituximab is not cost-effective compared with cyclosporine, given an ICER of \$368,823 per QALY gained. However, over a patient's lifetime rituximab has an ICER of \$41,947 per QALY gained making it more cost-effective than cyclosporine in the context of the commonly accepted US threshold of \$50,000 per QALY gained. Tacrolimus is more costly and less effective than cyclosporine and rituximab both after two years of treatment and over a patient's lifetime. **CONCLUSIONS:** Assuming the benefits of treatment persist over time, rituximab is more cost-effective than cyclosporine over a patient's lifetime but not after two years of treatment under both the standard US threshold of \$50,000 per QALY gained and an alternative higher threshold of \$100,000 per QALY gained. Additional research is needed to evaluate the long-term benefits of rituximab.

PSY40

COST MINIMIZATION ANALYSIS OF EQUIPOTENT OPIOID ANALGESICS: NATIONWIDE ANALYSIS OF MEDICARE PART D STAND-ALONE PRESCRIPTION DRUG PLANS

Li H, Patel R

University of the Pacific, Stockton, CA, USA

OBJECTIVES: Nearly 54 million Medicare beneficiaries, the overwhelming majority of whom are 65 plus years of age, reside in the US. Many beneficiaries suffer from chronic pain and need to use opioid analgesics for treatment. Medicare Part D is the outpatient prescription drug benefit available to beneficiaries through private insurance companies. Plan offerings differ across the country and each plan can have a different cost-sharing structure and formulary. We sought to examine the cost differences between therapeutically equivalent doses of opioid analgesics across all Medicare Part D Stand-alone prescription drug plans (PDPs). **METHODS:** Five short- and five long-acting opioid analgesic drugs were selected based on their prevalence of use and effectiveness at treating chronic pain. Therapeutically equivalent doses of each drug were entered into the Medicare Plan Finder Tool (www.medicare.gov) and 2015 data from 965 PDPs in 34 Medicare regions across the country were recorded. Collected data for each drug included: full cost, expected out-of-pocket costs both in a retail and mail order setting, and cost at a standard and preferred network pharmacy. **RESULTS:** Full annual retail drug costs varied from a low of \$134.42 (Methadone 5mg; thrice daily) to a high of \$10,443.30 (Hydromorphone ER 16mg; once daily). Beneficiary cost-sharing of the full annual drug costs in a retail pharmacy ranged from 36% (Hydrocodone 5/325; 12 tabs daily) to 93% (Hydromorphone ER). The annual out-of-pocket costs through mail order were between 88% (Oxycodone 5mg; 8 tabs daily) and 102% (Morphine 30mg; 2 tabs daily) of the costs in a retail pharmacy setting. Beneficiary out-of-pocket costs for the same drug was between 7% and 44% cheaper at a preferred network pharmacy as compared to a standard network pharmacy. **CONCLUSIONS:** Considerable cost variability exists among equipotent opioid analgesics across Medicare PDPs. Minimizing beneficiary out-of-pocket costs may improve economic outcomes without sacrificing clinical outcomes.

PSY41

FORECASTING THE UNITED STATES LIFETIME COST AND OUTCOMES OF IVACAFTOR IN PATIENTS WITH CYSTIC FIBROSIS

Dilokthornsakul P, Campbell JD

University of Colorado Anschutz Medical Campus, Denver, CO, USA

OBJECTIVES: Ivacaftor is a breakthrough treatment for cystic fibrosis (CF) patients with the G551D genetic mutation. Clinical trials show ivacaftor significantly improves lung function. Information on lifetime clinical effects and cost are lacking. This study aims to forecast lifetime outcomes and cost by comparing ivacaftor plus usual care versus usual care alone. **METHODS:** A lifetime Markov model of ivacaftor for G551D mutation CF patients aged ≥6 years was conducted from a United States payer perspective. The model consisted of 5 health states: 1) forced expiratory volume 1-second (FEV1) % predicted >70%, 2) 40%≤FEV1≤70%, 3) FEV1<40%, 4) Lung transplantation, and 5) Death. All inputs were determined by literature sources. Efficacy of ivacaftor was from previous randomized clinical trials for the first 2 years. The efficacy after 2 years was assumed half of the observed efficacy (consistent with United Kingdom assessment assumption). The budget impact was estimated. We indirectly estimated ivacaftor's improvement in CF outcome gaps compared to the non-CF population. **RESULTS:** Compared to the usual care alone, ivacaftor treatment was associated with 18.09 additional life-years [95% credible interval (CI); 14.63-21.13] and 14.92 additional quality-adjusted life-years (QALYs) [95%CI; 11.92 – 17.95] over an average lifetime. Moving from usual care alone to ivacaftor treatment was associated with reducing the survival and QALY gaps of the non-CF population by 52.32% and 44.29%, respectively. The incremental lifetime cost with 3% discount was \$3,740,480. The budget impact was \$0.09 per-member per-month [95% CI; \$0.07 to \$0.11]. **CONCLUSIONS:** Ivacaftor was forecasted to increase life-years and QALYs in CF patients with the G551D mutation and move morbidity and mortality outcomes closer to that of their non-CF peers. The overall cost in patients with ivacaftor is much higher than usual care, but comes at a relatively low budget impact. Uncertainty in this literature-informed analysis could be reduced with patient-level analyses.

PSY42

COST-UTILITY ANALYSIS OF PAIN MEDICATIONS USED TO TREAT ADULT PATIENTS WITH CHRONIC BACK PAIN IN THE UNITED STATES

Shah D, Anupindi VR, Vaidya V

University of Toledo, Toledo, OH, USA

OBJECTIVES: Chronic back pain is a common health problem which is associated with considerable costs in United States. The largest categories for pain therapy costs include NSAID analgesics and opioids. However there has been limited evidence outlining the effectiveness in terms of quality of life of patients taking these pain medications for treating chronic back pain. Objective of this study is to perform a cost-utility analysis of chronic back pain patients using NSAIDs versus those using opioids alone and/ or combination opioid analgesics. **METHODS:** This cross sectional, observational, cost utility study was conducted using the Medical Expenditure Panel Survey database. Adult's ≥ 18 years with chronic back pain diagnosis (identified by ICD-9 –CM codes) for atleast 3 consecutive rounds were included in the study. The study was conducted from a payer's perspective and only the direct costs were included. Utility was measured using SF-6D scores from the SF-12 questionnaire. **RESULTS:** 1340 chronic back pain patients were identified from 2012 MEPS database, of which 53% (n=704) were in NSAIDs group and 47% (n=636) were in opioids group. The total mean cost of NSAIDs group was found to be \$ 8005.3 while the total mean cost of opioids group was found to be \$12475.99. The QALYs of both the groups was almost the same with the mean QALY score of NSAIDs group being 0.645 and the opioids group being 0.615. **CONCLUSIONS:** Preliminary analysis showed that for the extra cost spent on Opioids, the effectiveness was seen to be almost the same as those taking NSAIDs. This may be because of the severe adverse events associated with the use of opioids which may reduce the quality of life of patients. Further research needs to be conducted by considering other pharmacological agents used to treat back pain and finding the most cost effective treatment.

PSY43

KIDNEY INVOLVEMENT IN TUBEROUS SCLEROSIS COMPLEX: THE IMPACT ON HEALTH CARE RESOURCE USE AND COSTS IN THE NETHERLANDS

Vekeman F¹, Magestro M², Karner P³, Duh M⁵, Nichols T³, Zonnenberg BA⁵

¹Group e d'analyse, Montreal, QC, Canada, ²Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA, ³Analysis Group, Boston, MA, USA, ⁴Analysis Group, Inc., Boston, MA, USA, ⁵University Medical Center Utrecht, Utrecht, The Netherlands

OBJECTIVES: Tuberous sclerosis complex (TSC) is a rare genetic disorder associated with angiomyolipomata (non-malignant kidney lesions) in the majority of patients. Angiomyolipomata increase in size over time, present risk of acute hemorrhage, and can lead to progressive chronic kidney disease (CKD). Our objective was to document the association between angiomyolipomata and CKD, including the impact on health care resource utilization (HCRU) and health care costs. **METHODS:** This retrospective, longitudinal cohort study used medical chart data from patients with TSC treated at a specialty center in the Netherlands from January 1990 to April 2012. Patients were followed longitudinally and classified into open cohorts based on their CKD stage (estimated from serum creatinine levels) and size and number of angiomyolipomata. Average glomerular filtration rates (GFR) and the proportions of patients reaching advanced CKD stages were compared with a non-TSC reference population. HCRU rates and health care costs (2012€) per patient per year (PPPY) were compared across cohorts. **RESULTS:** 369 patients were included (median [mean] follow-up time 15.4 [14.3] years). Compared with the non-TSC reference population, the decline in kidney function with age was steeper for patients with TSC (mean change in GFR/year = -1.53 vs. -0.94 mL/min/1.73 m²), and more patients with TSC reached CKD stage 3 or higher (16% vs. 3% of patients <70 years old). Compared with CKD stage 1, CKD stages 2 to 5 were associated with larger and more numerous angiomyolipomata, higher overall HCRU rates (rate ratios = 1.5 to 2.3, P < 0.01), and higher health care costs (incremental costs = €737 to €30,641 PPPY, P < 0.004). **CONCLUSIONS:** Our results suggest that impaired kidney function associated with angiomyolipomata imposes a significant burden and remains a key concern in patients with TSC. Treatments that slow the rate of kidney function decline in patients with TSC may substantially reduce the HCRU and costs associated with CKD and angiomyolipomata.

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

PSY44

CHARACTERISTICS OF COMBINATION PHARMACOTHERAPY IN PATIENTS WITH DIABETIC PAINFUL NEUROPATHY (DPN)

Kuo K, Brixner D, Lipman A, Hung M, Oederda S
University of Utah, Salt Lake City, UT, USA

OBJECTIVES: To compare patient characteristics between DPN patients receiving mono-pharmacotherapy and those receiving combination pharmacotherapy. **METHODS:** A patient cohort was identified diagnosed with DPN during 2006-2013 in Inovalon's MORE2® registry, a healthcare data warehouse with national medical/pharmacy claims, continuously enrolled for at least 18 months. Patients were included if they were ≥ 18 years at the time of their first DPN prescription for tricyclic antidepressant (TCAs), opioids, duloxetine, gabapentin, pregabalin, or lidocaine. They were classified as having mono- or combination pharmacotherapy (time between the first and second medicine was within 30 days). If there was 60-day prescription-filled gap, the prescription was classified as discontinued. Switch or add-on groups were categorized based on continuation of the index medicine. A simple proportional hazards model was conducted for comparing time to discontinuation, switch, or add-on. Multiple logistic regression was used for identifying predictors of combination pharmacotherapy. **RESULTS:** There were 7,145 patients on mono-pharmacotherapy, and 421 patients on combination pharmacotherapy. The top three index medicines were gabapentin (55.7%), opioids (13.1%), and pregabalin (12.9%) in the mono-pharmacotherapy group, and opioids+ gabapentin (27.1%), TCAs+ gabapentin (17.6%), and duloxetine+ gabapentin (8.6%) in combination group. Patients on combination pharmacotherapy were 57% less likely to discontinue than patients on mono-pharmacotherapy (95%CI = 0.34-0.54, p < 0.001), and 916% more likely to switch than patients on mono-pharmacotherapy (95%CI = 8.30-12.43, p < 0.001). There was no statistically significant difference in time to add-on (p = 0.069). Patients who are female, with >7 co-morbidities, and who had depression, or arthritis were more

likely to start with combination pharmacotherapy. Patients who are older than 65, and those who have hypertension were less likely to start with combination pharmacotherapy. **CONCLUSIONS:** Patients on combination were more likely to switch, less likely to discontinue than mono-pharmacotherapy. Age, gender, and co-morbidities were predictors of receiving combination pharmacotherapy in DPN patients.

PSY45

INITIAL IMPACT OF TELEPHARMACY ON SPECIALTY MEDICATION ADHERENCE

Fensterheim LE¹, Gunn JG¹, Pokuta KL², Straszewski A², Marks A¹

¹Catamaran, Chicago, IL, USA, ²Catamaran, Schaumburg, IL, USA

OBJECTIVES: Patients with specialty conditions are faced with a unique set of challenges in regard to being adherent to their medications. One-on-one pharmacist counseling from the comfort of the patient's home can be an innovative and impactful intervention that can lead to improved adherence for specialty patients. This study analyzes the impact on adherence after an initial video conferencing session between pharmacist and patient at the start of treatment. **METHODS:** Patients new to therapy receiving a video conferencing session from the BriovaRx® specialty pharmacy from April 1, 2013 – May 31, 2014 were included in the analysis. The comparison group consisted of non-BriovaRx® patients that were also new to therapy during this timeframe and continuously eligible from October 1, 2012 – November 30, 2014. The two groups of patients were matched on age, gender, and therapy. Adherence as measured by cumulative medication gap (CMG) was measured for both groups for 180 days after the start of treatment. The odds ratio was computed to assess the likelihood of a patient having an adherence greater than or equal to 80% with and without the video consultation. **RESULTS:** There were a total of 77 patients who received a video consultation and 117 total drug regimens. The distribution of disease states included: 25% hepatitis C, 22% rheumatoid arthritis, 18% multiple sclerosis and 35% other specialty conditions. The average age of patients receiving a consult was 46 with 56% female. The control group consisted of 1,465 patients and 1,736 total drug regimens. Patients had significantly higher odds of being adherent if they received a video consult (OR = 2.04; 95% CI, 1.02-4.07). **CONCLUSIONS:** This study highlights the benefit that video consultations can have on the adherence of specialty pharmacy patients. This is especially important given the high cost and complex management of specialty medications.

PSY46

UNMET NEEDS AND TREATMENT PATTERNS IN LUPUS: RESULTS FROM AN ONLINE COMMUNITY

Wang VC, Hanger M, Vaughan T, Woyczynski M, Wicks P
PatientsLikeMe, Cambridge, MA, USA

Wang V, Hanger M, Woyczynski M, Vaughan T, Wicks P
PatientsLikeMe, Cambridge, MA, USA. **OBJECTIVES:** (1) To understand the utility of using patient-reported data from an online Systemic Lupus Erythematosus (SLE) community for research, and (2) to understand the experience of living with SLE, including symptoms and treatments in a real-world environment. **METHODS:** Drawing on data captured by PatientsLikeMe, we explored reported patient symptoms, side-effects, treatments, and reasons for stopping treatment. We generated Kaplan-Meier curves of treatment duration for patients reporting particular side effects. Finally, to infer additional symptom and treatment factors that were important to SLE patients, we analyzed free text in patient forums and profiles. **RESULTS:** At the time of the research, PatientsLikeMe was being used by 5,714 SLE patients. With 2,570 PLM members with SLE (45%) reporting symptoms, lower back pain (53%), fatigue (52%), and joint pain (45%) were most likely to be rated as severe. In Forums, patients discuss pain, both general and localized, more than other frequently-measured symptoms, such as malar rashes. Of 5,026 members (88%) reporting treatments, hydroxychloroquine (73%), prednisone (63%), and OTC Naproxen (41%) were the most commonly used. Azathioprine, naproxen, and belimumab treatments were most often abandoned due to lack of efficacy, whereas hydroxychloroquine and methotrexate were more often abandoned due to side-effects. **CONCLUSIONS:** This study supports the ability of an online platform to capture important information on patient experience of disease and treatment, particularly in a poorly understood area such as lupus. Given the severity of side effects and abandonment of treatments, there is unmet need in SLE treatment. Clinical trials have often focused on easily measurable endpoints (e.g., rashes), but symptoms such as pain and fatigue are worthy of deeper study. Research that reflects the most common and severe symptoms will encourage development of higher-value lupus treatments.

PSY47

ESTIMATING UTILITY VALUES FOR POLYCYTHEMIA VERA USING PATIENT-REPORTED OUTCOMES FROM THE RESPONSE TRIAL AND EXISTING MAPPING ALGORITHMS

Thompson M¹, Pepper AN¹, Côté I², Oliver N³

¹Cornerstone Research Group, Burlington, ON, Canada, ²Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA, ³Novartis Pharmaceuticals UK Limited, Frimley/Camberley, Surrey, UK

OBJECTIVES: Utility values are required to conduct cost-utility analyses for the economic evaluation of novel therapies. While preference-based utility measures, such as the EQ-5D, are preferred by healthcare payers, they may inadequately capture condition-specific differences in health-related quality of life (HRQoL) or may not be available from clinical trials. Utility values for patients with polycythemia vera (PV), a myeloproliferative neoplasm (MPN), are not available in the literature; however, the PV RESPONSE trial collected EORTC QLQ-C30 and MPN-SAF data. The objective of the current study was to estimate utility values for PV patients using existing mapping algorithms. **METHODS:** EORTC QLQ-C30 data from the RESPONSE trial were mapped onto the EQ-5D using published mapping algorithms for various cancers (e.g., multiple myeloma, breast, gastric, esophageal). Utility values were also estimated using the MF-8D, a condition-specific preference-based measure that was developed for myelofibrosis, another MPN, using both the EORTC QLQ-C30 and MF-SAF. Utility values obtained from the various mapping algorithms were compared. **RESULTS:** At baseline in the RESPONSE trial, utility values calculated for PV patients using