Raising the Standard in HEOR
Analysis Group Posters and Presentations

ISPOR 22ND ANNUAL INTERNATIONAL MEETING, MAY 20-24, 2017

Analysis Group health outcomes professionals have extensive experience helping clients quantify product value in a dynamic and rapidly changing marketplace.

Drawing on our in-depth knowledge of a range of relevant data sources, we undertake U.S. and international pharmacoeconomic and health outcomes research and epidemiologic studies across a wide spectrum of therapeutic areas. Our work frequently results in publication in peer-reviewed journals. Together with prominent scholars from leading universities, we translate state-of-the-art academic theories into compelling results for our clients.

Analysis Group has 14 poster presentations and one educational symposium at the 2017 ISPOR meeting.

Please visit our team at Booth #804

ISPOR 2017 Analysis Group Symposium

Panel ID	Topic	Speakers	Abstract
MAY 23, SYMPOSIUM			**7:15 AM - 8:15 AM**
IP16	New Developments in Psychometric and Utility Methods for Patient-Reported Outcome Measurements in Health Economic Evaluations	Mei Sheng Duh (moderator), John E. Ware, Jr., John E. Brazier, PhD, Eric Q. Wu	Page 4

ISPOR 2017 Analysis Group Poster Presentations

Number	Topic	Authors	Abstract
MAY 22, POSTER SESSION I			**8:30 AM - 2:00 PM**
PIN47	A Claims-Based Analysis Of Hepatitis A, B, and A/B Vaccination Series Completion and Compliance Among US Adults RESEARCH PRESENTATION AWARD FINALIST	Wendy Y. Cheng, Emilie Duchesneau, Monica Macheca, Mei Sheng Duh	Page 4
MAY 22, POSTER SESSION II			**3:45 PM - 7:45 PM**
PCN17	Clinical Response and Time to Prostate-Specific Antigen (PSA) Progression in Patients with Metastatic Castration-Resistant Prostate Cancer (MCRPC) Receiving Second-Line Chemotherapy Versus Alternative Androgen Receptor-Targeted Agents (ARTA) after a Lack of Response to First-Line Arta in US Community Oncology Practices	Wendy Y. Chen, Mei Sheng Duh	Page 5
PCN31	Real-World Outcomes Among Patients Who Initiated Pazopanib or Sunitinib as First Targeted Therapy for Advanced Renal Cell Carcinoma (aRCC): A Retrospective Analysis of Medicare Data	Elyse Swallow, Miranda Peeples, Miriam Zichlin, Mark Meiselbach	Page 5
PCN32	A Meta-Analysis and Meta-Regression of the Efficacy of Front-Line Treatment Combinations with Ponatinib Versus 1st- and 2nd-Generation Tyrosine Kinase Inhibitors for Ph+ Acute Lymphoblastic Leukemia	Maral DerSarkissian, Mei Sheng Duh, Nora McCormick, Wendy Y. Cheng, Ariadne Souroutzidis	Page 6
PCN65	Assessment of Costs Associated with Adverse Events in Patients with Cancer	Martin Cloutier, Marjolaine Gauthier-Loiselle, Patrick Gagnon-Sanschagrin, Annie Guérin	Page 6
PCN95	Real-World Treatment Patterns and Costs in Medicare Beneficiaries Newly Diagnosed with Acute Myeloid Leukemia	Dominick Latremouille-Viau, Annie Guérin, Sherry Shi, Patrick Gagnon-Sanschagrin	Page 7
PCN190	Assessment of the Association Between the Burden of Carcinoid Syndrome Symptoms and the Quality of Life Among Patients with Carcinoid Syndrome in the United States Based on the Fact-G Instrument RESEARCH PRESENTATION AWARD FINALIST	Lynn Huynh, Todor Totev, Mei Sheng Duh, Rachel Bhak	Page 7
PMS71	Association Between Disease Activity Level Improvement and Reduction in Healthcare Resource Utilization in Patients with Rheumatoid Arthritis Receiving Targeted Therapy	Andrea Lopez, Mei Sheng Duh, Erin McDonnell, Priyanka Bobbili, Batool Haider	Page 8

ISPOR 2017 Analysis Group Poster Presentations

Number	Topic	Authors	Abstract
MAY 23, POSTER SESSION III			**8:30 AM - 2:00 PM**
PND22	Healthcare Costs Among Patients with Lennox-Gastaut Syndrome Treated with Clobazam	Mei Sheng Duh, Wendy Y. Chen, Edward Tuttle	Page 10
PND45	Patient Characteristics and Treatment Adherence Among Patients Treated with Delayed-Release Dimethyl Fumarate for Relapsing Remitting Multiple Sclerosis in Israel	Urvi Desai, Noam Kirson, Michael Hellstern, Sophie Schonfeld	Page 11
PSY81	Assessing Utility Values for Treatment-Related Health States of Acute Myeloid Leukemia in the United States	Min Yang, Wei Gao, Philip Galebach, Cheryl Q. Xiang	Page 11
PSY88	Value of Transfusion Independence in Severe Aplastic Anemia from Patients' Perspectives - A Discrete Choice Experiment RESEARCH PRESENTATION AWARD FINALIST	Lynn Huynh, Jasmina I. Ivanova, Todor Totev, Sophia Graham, Mei Sheng Duh	Page 12
PSY128	Disease-Stratified Adherence to Iron Chelation Therapy in Patients Who Switched from Deferasirox Dispersible Tablets to Deferasirox Film-Coated Tablets	Wendy Y. Chen, Mei Sheng Duh, Yongling Xiao	Page 12
MAY 23, POSTER SESSION IV			**3:45 PM - 7:45 PM**
PMH27	Healthcare Resource Utilization and Costs Among Adult Schizophrenia Patients Using Once-Monthly Versus Twice-Monthly Long-Acting Antipsychotics RESEARCH PRESENTATION AWARD FINALIST	Dominic Pilon, Yongling Xiao, Philippe Thompson-Leduc, Patrick Lefebvre	Page 13

ISPOR 2017 Analysis Group Abstracts

EDUCATIONAL SYMPOSIUM

MAY 23, 7:15 AM - 8:15 AM

SYMPOSIUM
NEW DEVELOPMENTS IN PSYCHOMETRIC AND UTILITY METHODS FOR PATIENT-REPORTED OUTCOME MEASUREMENTS IN HEALTH ECONOMIC EVALUATIONS

Description: The necessity of having valid, sensitive, and reproducible patient-reported outcome (PRO) tools to capture the benefits of treatments has never been greater because of the importance of health economic and outcomes evaluations of medical interventions. This 3-part symposium begins with a summary of lessons (old and new) that are at the foundation of conceptualizing, and quantifying descriptions of health-related quality of life. Lessons include the essential domains of health, the sometimes substantial implications of different operational definitions for each health factor (domain), and advances in measuring disease-specific PROs using standard content and scoring across conditions. This will be followed by describing parallel developments in the utility field, such as developing condition-specific preference-based measures and how they compare to generic utility measures, extending generic utility measures to incorporate higher levels of functional health and well-being, and calibrating different measures through linking and mapping. Lastly, some of the challenges in valuation will be elaborated with discussion of how to apply new developments in utility and statistics to address these challenges.

POSTER SESSION I

MAY 22, 8:30 AM - 2:00 PM
Poster Discussion Hour 1:00 PM - 2:00 PM

PIN47
A CLAIMS-BASED ANALYSIS OF HEPATITIS A, B, AND A/B VACCINATION SERIES COMPLETION AND COMPLIANCE AMONG US ADULTS
RESEARCH PRESENTATION AWARD FINALIST

Objectives: Literature on guideline adherence for hepatitis A, B, and A/B vaccines is limited. We assessed hepatitis A, B, and A/B vaccination completion and compliance rates among US adults.

Methods: Data were from Truven Marketscan commercial/Medicare and Medicaid healthcare claims (Q1 2007-Q3 2015). Patients had ≥1 claim for a hepatitis A, B, or A/B vaccine, ≥19 years at first claim ("index date"), ≥12 months of enrollment pre-index date, ≥18 months of enrollment post-index date, and <2 hepatitis A or B diagnoses pre-index date. Using CDC guidelines, we defined completion as receiving the correct number of doses separated by minimum intervals and compliance as receiving the correct number of either monovalent or bivalent vaccines within the product labels' specified timeframes. % patients completing and complying with the hepatitis A and B series were presented with % patients completing stratified by monovalent versus bivalent vaccine as initial dose.

Results: 395,323 commercial/Medicare and 13,822 Medicaid patients were included. Patients were on average 43.4 years old and mostly female. Among commercial/Medicare patients with a monovalent-vaccine initial dose, completion rates were 32.0% and 39.6% for hepatitis A and B, while 65.1% received at least a second dose of hepatitis B vaccine. Among patients initiating with a bivalent vaccine, 47.3% and 47.2% completed the hepatitis A and B series, with approximately 74.6% receiving at least two doses of hepatitis B vaccine. Among Medicaid patients, completion rates for hepatitis A, B, and A/B vaccines ranged from 21.0%-24.1%. Compliance rates for hepatitis A, B, and A/B vaccines were 27.9%, 12.2%, and 23.9% in commercial/Medicare patients, and 18.4%, 5.6%, and 9.9% in Medicaid patients.

Conclusions: Adult completion and compliance rates for hepatitis A, B, and A/B vaccines are suboptimal. Research investigating completion and compliance predictors is needed.

POSTER SESSION II

MAY 22, 3:45 PM - 7:45 PM

Poster Discussion Hour 6:45 PM - 7:45 PM

PCN17

CLINICAL RESPONSE AND TIME TO PROSTATE-SPECIFIC ANTIGEN (PSA) PROGRESSION IN PATIENTS WITH METASTATIC CASTRATION-RESISTANT PROSTATE CANCER (MCRPC) RECEIVING SECOND-LINE CHEMOTHERAPY VERSUS ALTERNATIVE ANDROGEN RECEPTOR-TARGETED AGENTS (ARTA) AFTER A LACK OF RESPONSE TO FIRST-LINE ARTA IN US COMMUNITY ONCOLOGY PRACTICES

Objectives: The relationship between treatment sequence and outcomes in mCRPC is unclear. This retrospective cohort study assessed if second-line (2L) taxane-based chemotherapy vs alternative ARTA is associated with improved clinical response and time to PSA progression in patients with a lack of response to first-line ARTA in the US community oncology setting.

Methods: Using Altos electronic medical records, 345 mCRPC patients were identified who lacked response to first-line ARTA (abiraterone: N=289; enzalutamide: N=56) and received 2L chemotherapy (docetaxel: N=128; cabazitaxel: N=19), or alternative ARTA (enzalutamide: N=170; abiraterone: N=28) from 05/2011 to 10/2014. Outcomes were evaluated from second-line therapy initiation and compared between the two cohorts using one-sided tests. Clinical response (clinical note, ECOG performance status (PS) reduction by ≥1, ≥5% weight increase, or ≥2g/dl hemoglobin (Hb) increase over ≥3 months) and time to PSA progression (≥25% increase over nadir concentration) were assessed using logistic and Cox regressions adjusted for year, age, metastases, opioid use, ECOG PS, PSA, Hb, alkaline phosphatase (ALP), lactate dehydrogenase (LDH), and albumin (Alb) levels.

Results: At start of 2L therapy, patients receiving chemotherapy vs ARTA were younger (median age, 74 vs 79 years) and had a poorer prognosis: higher mean PSA (439 vs 231 ng/mL), LDH (344 vs 234 µg/L) and ALP (241 vs 166 µ/L) levels, lower mean Hb levels (11 vs 12 g/dL), higher mean Halabi risk score (159 vs 137; JCO 2014:32;671–7), and more patients had Alb levels <lower limit of normal (25% vs 15%); all p <0.01. Patients in the chemotherapy vs ARTA cohort were more likely to have a clinical response (adjusted odds ratio=1.78, p=0.020) and longer time to PSA progression (adjusted hazard ratio=0.66, p=0.010).

Conclusions: 2L taxane-based chemotherapy vs 2L ARTA may be more suitable for patients with a lack of response to first-line ARTA and therefore should be further investigated in a prospective randomized trial.

PCN31

REAL-WORLD OUTCOMES AMONG PATIENTS WHO INITIATED PAZOPANIB OR SUNITINIB AS FIRST TARGETED THERAPY FOR ADVANCED RENAL CELL CARCINOMA (ARCC): A RETROSPECTIVE ANALYSIS OF MEDICARE DATA

Objectives: This study assessed real-world overall survival (OS), time on treatment (TOT), and dose intensity among aRCC patients who initiated pazopanib or sunitinib, two commonly-used first targeted therapies (TT).

Methods: Patients aged ≥65 with aRCC who initiated pazopanib or sunitinib as first TT (index date) were identified from the 100% Medicare data + Part D linkage (1/1/2006-12/31/2014). Patients were stratified by first TT and matched 1:1 using propensity scores based on age, sex, race, year of RCC diagnosis, metastatic sites, and baseline comorbidities and costs (assessed 1 year before index date). OS was defined as the time from index date to death from any cause; TOT as the time from index date to the earliest of treatment discontinuation (a prescription gap of >90 days) or death from any cause. For both outcomes, patients were censored at the earliest of end of eligibility or data cut-off. Dose intensity was defined as the ratio of days that the patient had received drug supply to TOT. OS and TOT were compared between matched cohorts using Kaplan-Meier analyses and univariable Cox models; dose intensity was compared using Wilcoxon signed-rank tests.

Results: Before matching, the pazopanib cohort (N=526) was associated with higher outpatient visits and costs and lower pharmacy costs than the sunitinib cohort (N=1,185; all p<0.05). After matching, all baseline characteristics were balanced (N=522 for both). First TT with pazopanib was associated with significantly longer OS (median: 18.2 vs. 14.6 months, p<0.05; hazard ratio [HR]=0.83, 95% confidence interval [CI]: 0.72-0.97), similar TOT (median: 4.8 vs. 4.1 months, p=0.16; HR=0.90, 95% CI: 0.78-1.04), and lower dose intensity (mean: 0.91 vs. 0.94, p<0.01) compared with the sunitinib cohort.

Conclusions: Among Medicare patients with aRCC, first TT with pazopanib compared to sunitinib was associated with significantly longer OS, similar TOT, and lower dose intensity.

PCN32
A META-ANALYSIS AND META-REGRESSION OF THE EFFECTIVENESS OF FRONT-LINE TREATMENT COMBINATIONS WITH PONATINIB VERSUS 1ST- AND 2ND-GENERATION TYROSINE KINASE INHIBITORS FOR PH+ ACUTE LYMPHOBLASTIC LEUKEMIA

Objectives: To compare the effectiveness, as measured by complete molecular response (CMR) and 2- and 3-year overall survival (OS), of ponatinib versus first- and second-generation TKIs (i.e., imatinib, dasatinib, and nilotinib) for treatment of de novo Philadelphia-positive acute lymphoblastic leukemia (Ph+ ALL).

Methods: Twenty-six studies of front-line Ph+ ALL treatment with a TKI in combination with chemotherapy or corticosteroids were identified from published targeted literature reviews and recently published trials. Study arms in which patients received chemotherapy or corticosteroids only, a single TKI agent, or autologous stem cell transplant exclusively, were excluded. The proportions of patients achieving CMR (no detectable BCR-ABL1 transcripts) and 2- and 3-year OS were extracted from all study arms and summarized by TKI group (ponatinib versus earlier-generation TKIs) using pooled estimates with 95% confidence intervals (CIs) from a random-effects meta-analysis. Multivariate logistic meta-regressions adjusting for age and gender estimated the association between TKI-treatment group and percent

CMR, 2-year OS, and 3-year OS. Odds ratios (OR) and 95% CIs were reported.

Results: Thirty-two TKI treatment arms were analyzed. The pooled proportion of patients achieving CMR with ponatinib was higher than that with earlier-generation TKIs (79% versus 34%). The pooled estimates of 2- and 3-year OS were also higher with ponatinib than with earlier-generation TKIs (2-year: 83% versus 58%; 3-year: 79% versus 50%). The OR for ponatinib versus earlier-generation TKIs for CMR (N=25) was 6.09 (95% CI: 1.16-31.90, p=0.034); for 2-year OS (N=27) 3.70 (95% CI: 0.93-14.73, p=0.062); for 3-year OS (N=19) 4.49 (95% CI: 1.00-20.13, p=0.050).

Conclusion: Compared to earlier-generation TKIs, ponatinib was associated with a >6-fold, >3-fold, and >4-fold increase in the odds of achieving CMR, 2-year OS, and 3-year OS, respectively. Ponatinib in combination with chemotherapy may represent an effective front-line treatment option in newly diagnosed Ph+ ALL compared with combination therapy with earlier-generation TKIs.

PCN65
ASSESSMENT OF COSTS ASSOCIATED WITH ADVERSE EVENTS IN PATIENTS WITH CANCER

Background: This study assessed the incremental costs associated with adverse events (AEs) in a range of malignancies.

Methods: Using Truven Health Analytics MarketScan® databases (2000:Q1-2015:Q3), patient-level treatment episodes for breast, gastrointestinal, genitourinary, lung, hematologic, and skin cancers were identified. Based on current National Comprehensive Cancer Network Treatment Guidelines, 104 prescribing labels were reviewed to identify 36 AEs of interest. Episodes with a claim for an AE were matched with episodes without the AE on a 1:1 ratio based on demographics, insurance plan type, therapy line, treatment regimen, cancer characteristics, and episode duration. Healthcare costs (2015 USD) were compared between episodes with and without each AE using multivariate generalized linear regression models adjusting for the presence of other AEs.

Results: A total of 794,243 episodes were identified; mean patient age was 62.8 years; 58.1% were female; and 45.3% were first, 24.3% second, and 30.4% third or

later line therapy following primary diagnosis. The number of matched episodes for each AE ranged from 878 to 115,754, with mean duration ranging from 4.7 to 16.4 months. The most prevalent AEs were pain (prevalence: 28.2%; incremental adjusted costs per episode: $4,576), hypertension (27.5%; $2,416), anemia/pallor (17.8%; $4,826), psychiatric disorders (13.9%; $3,458), and cough/upper respiratory infections (13.6%; $393); all p<0.05. The most costly AEs were central nervous system hemorrhage (0.2%; $26,904), sepsis/septicemia (2.5%; $25,562), gastrointestinal perforation (0.2%; $24,141), pancreatitis (0.1%; $17,987), and gastrointestinal fistula (0.1%; $15,881); all p<0.05.

Conclusions: The prevalence and cost of AEs in patients with cancer tended to have an inverse relationship, with some of the most prevalent AEs being less costly and some of the most costly AEs being fairly rare. Treatment AEs may add a significant amount of cost to a treatment. Cancer therapies that are well tolerated are needed to further reduce the economic burden on patients and the health care system.

PCN95

REAL-WORLD TREATMENT PATTERNS AND COSTS IN MEDICARE BENEFICIARIES NEWLY DIAGNOSED WITH ACUTE MYELOID LEUKEMIA

Background: Little is known on treatment patterns and costs of acute myeloid leukemia (AML) management in US clinical practice. This study describes induction therapy and consolidation cycles in terms of settings, duration, and costs of Medicare beneficiaries with AML who are candidates for standard chemotherapy.

Method: Using the SEER-Medicare databases, Medicare beneficiaries newly diagnosed with AML from 2007-2011 who received standard induction chemotherapy in an inpatient setting were selected. Patients were observed from induction therapy initiation to the first event among hematopoietic stem cell transplant, death, end of Medicare coverage/data availability, or 180-days after the end of the induction episode. AML treatment episodes, including induction therapy and consolidation cycles, were identified using DRG/procedure codes. AML treatment episode settings, duration, and costs (USD2015, public payers' perspective) were analyzed.

Results: Of the 563 Medicare beneficiaries (mean age=66 years; 54% male) with a first induction episode, 193 (34%) patients had 2 cycles of induction therapy during this episode. The median duration of inpatient stays with 1 cycle of induction therapy was 28 days and mean costs were $64,680. The median duration of inpatient stays with 2 cycles of induction therapy was 47 days and mean costs were $126,096.

Following induction therapy, 297 (53%) patients had ≥1 consolidation cycle, 231 (40%) ≥2, 148 (26%) ≥3, and 87 (15%) ≥4. 65% of consolidation cycles were administered in an inpatient setting and 35% in an outpatient setting. In the inpatient setting, the median duration per cycle was 6 days and mean costs were $28,843. In the outpatient setting, the median duration per cycle was 5 days and mean costs were $5,803.

Conclusions: This is the first exploratory study reporting recent treatment patterns and costs of Medicare beneficiaries newly diagnosed with AML. These findings suggest that there is substantial heterogeneity in the consolidation therapy setting and costs.

PCN190

ASSESSMENT OF THE ASSOCIATION BETWEEN THE BURDEN OF CARCINOID SYNDROME SYMPTOMS AND THE QUALITY OF LIFE AMONG PATIENTS WITH CARCINOID SYNDROME IN THE UNITED STATES BASED ON THE FACT-G INSTRUMENT
RESEARCH PRESENTATION AWARD FINALIST

Objectives: To assess the association between the burden of carcinoid syndrome symptoms (CSS) and quality of life (QoL) among patients with carcinoid syndrome using the validated Functional Assessment of Cancer Therapy-General (FACT-G) instrument.

Methods: Patients with CSS in the US were recruited via Neuroendocrine Cancer Awareness Network for an online, anonymous survey between July and October 2016. Eligible patients were at least 18 years old with CSS and received either somatostatin analogs (SSA) or non-SSA treatments for CSS control. The survey consisted of demographic, clinical, and QoL questions, including the FACT-G questionnaire. Descriptive and multivariable regression analyses, adjusting for demographic and clinical

characteristics, were performed to assess the association between CSS and total FACT-G score.

Results: Among 117 patients with CSS, who completed the survey, 76.9% were female and 87.2% were Caucasian with a mean age of 58.0 years. Patients reported experiencing up to 6 CSS (mean±SD: 3.0±1.1) after diagnosis with neuroendocrine tumor. Carcinoid diarrhea (97.4%) and flushing (90.6%) were the most common CSS. Majority of patients (98.3%) reported receiving SSAs in the past month, and the mean±SD FACT-G total score was 67.6±20.0 (possible range: 0-108), which is lower than the general US population (80.1±18.1). Descriptive analysis suggested that FACT-G total score and subdomain scores were negatively associated with CSS burden. Multivariable models revealed that the FACT-G total score was decreased by 3.4 points (P=0.034) for each additional CSS, ≥4 bowel movements/day was associated with a 7.1 point decrease in FACT-G total score as compared to having <4 bowel movements/day (P=0.043), and that reduced activity levels (bed rest at <50% or ≥50% of the day, compared to normal activity) decreased the FACT-G total score by 25.4 and 35.5 points, respectively (both P<0.001).

Conclusions: This study suggests that CSS burden and impaired activity level are associated with lower QoL among patients with carcinoid syndrome.

PMS71

ASSOCIATION BETWEEN DISEASE ACTIVITY LEVEL IMPROVEMENT AND REDUCTION IN HEALTHCARE RESOURCE UTILIZATION IN PATIENTS WITH RHEUMATOID ARTHRITIS RECEIVING TARGETED THERAPY

Objectives: To evaluate the impact of change in patient-reported disease activity assessment measured using the Routine Assessment of Patient Index Data 3 (RAPID3) on healthcare resource utilization (HRU) in patients with rheumatoid arthritis (RA) receiving targeted therapy.

Methods: Electronic medical records (EMR) from Reliant Medical Group (Worcester, MA, USA) were used to identify adult RA patients with first observed prescription for a targeted therapy ("index") between 1/1/2008 and 6/30/2015. Change in RAPID3 score was calculated as the difference in RAPID3 score (range 0-30) at baseline and 6 (±1) months post-index and was categorized as good, moderate or poor response. All-cause and RA-related medical visits, including inpatient, emergency room, and outpatient visits, as well as prescription drug use were assessed using EMR data in the 6-12 month period following index. A generalized linear model with a log link function and negative binomial distribution was used to assess the association between RAPID3 response category and HRU outcomes, adjusting for RAPID3 score and HRU at baseline.

Results: The mean age of the study population (N=90) was 59.6 years and 73.3% were female. The mean RAPID3 score was 13.2 at baseline and 10.1 at 6 months post-index; 41 patients (45.6%) had good (N=24) or moderate (N=17) disease response. Compared to patients with poor response, patients with good/moderate response had fewer mean all-cause medical visits (6.1 vs. 11.0, p=0.003), RA-related medical visits (2.5 vs. 5.3, p=0.022), and non-targeted therapy prescriptions (7.3 vs. 12.2, p=0.006). Multivariate regression models showed patients with good/moderate response had significantly reduced all-cause (incidence rate ratio [IRR]=0.68, p=0.029) and RA-related (IRR=0.59, p=0.008) visits, driven mainly by reduction in outpatient visits, compared to poor responders.

Conclusions: Patients with good or moderate disease response to targeted therapy, as assessed by RAPID3, have reduced HRU, highlighting the association of patient-reported disease response and economic endpoints.

POSTER SESSION III

MAY 23, 8:30 AM - 2:00 PM
Poster Discussion Hour 1:00 PM - 2:00 PM

PND22
HEALTHCARE COSTS AMONG PATIENTS WITH LENNOX-GASTAUT SYNDROME TREATED WITH CLOBAZAM

Objectives: This retrospective analysis of healthcare claims data was conducted to examine healthcare costs in patients with probable Lennox-Gastaut syndrome (LGS) treated with clobazam (CLB).

Methods: Patients with likely LGS were identified from epilepsy patients (≥2 claims for epilepsy [ICD-9-CM 345.xx] or unspecified epilepsy [ICD-9-CM 780.39]) in 6 state Medicaid databases using a random forest machine-learning algorithm. CLB users were defined as having ≥2 prescription claims for CLB and ≥1 year of continuous enrollment before CLB initiation (index date). Monthly healthcare costs were estimated for pre-CLB (1 year before index date) and post-CLB (to end of data availability) periods. Generalized estimating equations were used to characterize and predict time trends before and after CLB initiation. To estimate the impact of CLB use on healthcare costs, the slope of monthly costs before CLB initiation was extrapolated beyond the index date and compared to post-CLB costs. The difference was quantified by calculating areas under the curve (AUCs).

Results: A total of 1,301 likely LGS patients were identified. Mean (SD) duration of observation post-CLB initiation was 1.60 (0.86) years. When compared to extrapolated costs without CLB during the post-CLB period, CLB treatment was associated with significant reductions in total all-cause, total epilepsy-related, and inpatient medical costs (P<0.05), and a nonsignificant increase in home care costs (P=0.45). The difference in AUCs was equivalent to a reduction of $1,538.89 (3.80%) in all-cause healthcare costs in the year after CLB initiation, and reductions of $2,236.34 (11.99%) and $3,112.29 (26.66%) for total epilepsy-related and inpatient costs, respectively. The cost increase for home care was $1,839.74 (11.62%).

Conclusions: Results from this study indicate that CLB initiation in LGS patients is associated with reduced healthcare costs, mainly driven by lower inpatient costs.

PND45
PATIENT CHARACTERISTICS AND TREATMENT ADHERENCE AMONG PATIENTS TREATED WITH DELAYED-RELEASE DIMETHYL FUMARATE FOR RELAPSING REMITTING MULTIPLE SCLEROSIS IN ISRAEL

Objectives: Few studies have relied on real-world data to describe the characteristics and treatment patterns of patients using oral disease-modifying treatments (DMTs) for relapsing remitting multiple sclerosis (RRMS) outside the US. This study reports the demographics, MS symptom burden, and treatment adherence among patients initiating treatment with a leading oral DMT – delayed-release dimethyl fumarate (DMF, also known as gastro-resistant DMF) – in Israel.

Methods: Patients aged ≥18 years diagnosed with MS and with ≥1 prescription for DMF were selected from de-identified electronic medical records data from a health fund in Israel (1/2013-1/2016). Patients were required to have continuous enrollment with the health fund for ≥12 months before (baseline) and ≥6 months after (follow-up) the first observed prescription (index) date. Patient characteristics during baseline and adherence during the follow-up period, as measured by medication possession ratio (MPR), were described.

Results: Of the 177 patients meeting the selection criteria (mean age: 40 years; ~30% male), 114 (64%) had previously used injectable DMTs for RRMS (primarily interferon beta). The average duration from MS diagnosis to index date was 69 months (median: 45 months). Nearly half of the patients had indications of neuropathic or musculoskeletal pain in the year before index, 26% bladder or bowel problems, 16% vision issues, 15% cognitive or psychiatric disorders, and 15% depression. The mean MPR during the 6-month follow-up period was 0.81 (median: 0.91); over two-thirds (69%) of the patients were considered adherent (MPR >0.8).

Conclusions: Patients initiating DMF in Israel had considerable symptom burden, and many used injectable DMTs prior to treatment initiation, possibly reflecting the relatively recent approval of the medication in Israel. Most patients were adherent to treatment during the 6-month follow-up period. Further research is needed to understand long-term adherence among Israeli patients.

PSY81
ASSESSING UTILITY VALUES FOR TREATMENT-RELATED HEALTH STATES OF ACUTE MYELOID LEUKEMIA IN THE UNITED STATES

Objectives: Preference valuations of health status are essential in health technology and economic appraisal. This study estimated utilities for treatment-related health states of acute myeloid leukemia (AML) and dis-utilities of severe adverse events (SAEs) using a sample of individuals representative of the adult general population of the United States.

Methods: Applying the discrete choice experiment (DCE) methodology, an online survey was designed to capture the preference responses for pairs of health status scenarios including treatment-related AML health states and key grade 3/4 AEs of chemotherapies. Treatment-related AML health states, developed based on literature review and interviews with clinicians, included complete remission (CR), no CR, relapse, stem cell transplant (SCT), and post SCT short-term recovery. Six attributes, including fever, lack of energy, problems with daily function, anxiety/depression, blood transfusions, and hospitalization, each with 2 to 4 varying levels were used to define health states. Coefficients from conditional logistic regression model with generalized estimating equations were used to generate utilities of the predefined health states and dis-utilities of SAEs.

Results: 300 respondents completed the survey. The distribution of respondent demographics (age, race, gender, region, and income) was within a 3% margin of the distribution reported in the 2010 US Census data. CR had the highest utility value (0.875), followed by post SCT short-term recovery (0.398), relapse (0.355), no CR (0.262), and SCT (0.158). Of the key AEs, serious infection leads to the highest decline in utility by 0.218, followed by severe diarrhea (0.176), abnormally low blood cell counts (0.100), and redness/skin peeling (0.060).

Conclusions: AML and treatments can be associated with a reduced quality of life and impaired ability to perform daily activities. The level of impairment depends on health status, including SAEs experienced during treatment. Findings from this study supported DCE methodology as a valid approach to obtain societal preference values for treatment-related health states.

PSY88
VALUE OF TRANSFUSION INDEPENDENCE IN SEVERE APLASTIC ANEMIA FROM PATIENTS' PERSPECTIVES – A DISCRETE CHOICE EXPERIMENT
RESEARCH PRESENTATION AWARD FINALIST

Objectives: Aplastic anemia is a rare (600-900 US cases/year), serious blood disorder due to bone marrow failure to produce blood cells. Transfusions are used to reduce risk of bleeding, infection, and relieve anemia symptoms. In severe patients, transfusions may be required more than once/week. The study aimed to elicit patient preferences for attributes associated with severe aplastic anemia (SAA) treatment, including transfusion independence.

Methods: An online discrete choice experiment (DCE) was conducted among patients with SAA who experienced insufficient response to immunosuppressive therapy (IST) and transfusion dependence for ≥3 months in the past 2 years. Recruitment occurred through an International Foundation and clinical sites. The DCE elicited preferences between hypothetical treatment pairs characterized by a common set of attributes: transfusions frequency, fatigue, risk of infection, and risk of serious bleeding. Conditional logit model with effects coding was used to estimate part-worth utilities for different attribute levels and assess the relative importance of each attribute. Predicted utility scores for transfusion frequency levels were reported.

Results: Thirty patients completed the survey. Most were age ≥40 years (73%), female (70%), and from the US (87%). 33% underwent bone marrow transplant; 37% received iron chelation therapy. Patients largely agreed that transfusion independence would bring less burden on time and costs, greater control and quality of life, less fatigue (87% noted each) and less scheduling around medical appointments (83%). The DCE found highest relative importance for risk of bleeding (0.30), followed by risk of infection (0.28), fatigue (0.23), and frequency of transfusions (0.20). More frequent transfusions resulted in lower utility, particularly increasing monthly transfusions frequency from 4 (0.57) to 8 (0.35).

Conclusions: Among SAA patients with insufficient response to IST, estimated utility was higher with fewer transfusions. While risk of bleeding, risk of infection, and fatigue were more important for patient treatment preferences, frequency of transfusions was also important.

PSY128
DISEASE-STRATIFIED ADHERENCE TO IRON CHELATION THERAPY IN PATIENTS WHO SWITCHED FROM DEFERASIROX DISPERSIBLE TABLETS TO DEFERASIROX FILM-COATED TABLETS

Objectives: To assess real-world adherence and persistence to iron chelation therapy (ICT) in patients switching from deferasirox (DFX) dispersible tablet (DFX-DT) to film-coated tablet (DFX-FCT), stratified by disease.

Methods: A retrospective pre-post cohort study was conducted in patients switching from DFX-DT to DFX-FCT using pharmacy/medical claims (06/2014 - 05/2016) from the Symphony Health Solutions' Integrated Dataverse (IDV®) database. Eligible patients were ≥2 years old, had a diagnosis of sickle cell disease (SCD), thalassemia, or myelodysplastic syndrome (MDS), ≥2 DFX-FCT claims (1st claim = index date), and ≥2 DFX-DT claims pre-index. Medication possession ratio (MPR) (percentage of time with access to medication) was computed for DFX-DT during the DFX-DT period (1st DFX-DT claim to index date) and for DFX-FCT during the DFX-FCT period (index date to end of observation). Proportion of days covered (PDC) and persistence (without a gap ≥30, 60 days) were assessed over DFX-DT- and DFX-FCT- periods of 3 and 6 months. Comparisons between the two periods were made using Wilcoxon sign-rank tests or McNemar's tests.

Results: A total of 348, 154, and 106 patients with SCD, thalassemia, and MDS were identified, respectively. Of all patients, 55% were female and 66% aged <35 years. Across all diseases, PDC and persistence to ICT were consistently higher during the DFX-FCT vs. DFX-DT periods, with the greatest improvement observed among MDS patients: DFX-FCT vs. DFX-DT, mean 3-month PDC: SCD 0.80 vs. 0.68, thalassemia 0.85 vs. 0.77, MDS 0.86 vs. 0.69; 3-month persistence: SCD 82.9% vs. 57.3%, thalassemia 90.3% vs. 71.8%, MDS 91.9% vs. 62.9% (all p<0.01). MPR was significantly different only in MDS patients (DFX-FCT: 0.88 vs. DFX-DT: 0.82, p=0.05).

Conclusions: Adherence and persistence to ICT improved significantly after patients with SCD, thalassemia, or MDS switched from DFX-DT to DFX-FCT, with the most notable improvements seen in patients with MDS.

POSTER SESSION IV
MAY 23, 3:45 PM - 7:45 PM
Poster Discussion Hour 6:45 PM - 7:45 PM

PMH27
HEALTHCARE RESOURCE UTILIZATION AND COSTS AMONG ADULT SCHIZOPHRENIA PATIENTS USING ONCE-MONTHLY VERSUS TWICE-MONTHLY LONG-ACTING ANTIPSYCHOTICS
RESEARCH PRESENTATION AWARD FINALIST

Objectives: To compare real-world healthcare resource utilization (HRU) and costs between schizophrenia patients stabilized on once monthly (OM) second generation long-acting injectable antipsychotic (LAI) versus twice-monthly (TM) second generation LAI.

Methods: Medicaid data from 6 states were used to identify adults with schizophrenia. Patients with ≥2 consecutive claims of the same OM LAI (paliperidone palmitate or aripiprazole) or TM LAI (risperidone) within 45 days with the same dosage and days supplied were selected. Patients needed ≥6 months of eligibility prior to LAI initiation and were observed from the second consecutive claim (index date) to the end of data availability. Outcomes were measured for 12 months after the index date. HRU was compared using incidence rate ratios (IRRs) and 95% confidence intervals (95%CIs) from multivariate generalized linear regression models with a negative binomial distribution. Costs were compared using linear regressions, with pvalues estimated using bootstrap techniques with resampling (B=499).

Results: A total of 785 OM patients and 625 TM patients met all study criteria. Patients in the OM cohort were younger (40 vs. 42 years, p=0.022) and were more likely to be men (68% vs. 63%, p=0.043) than in the TM cohort. After adjustment for potential confounders, patients in the OM cohort had fewer outpatient visits (IRR: 0.89, 95%CI: 0.79; 1.00), inpatient visits (IRR: 0.73, 95%CI: 0.58; 0.92), and longterm care visits (IRR: 0.58, 95%CI: 0.35; 0.94). There was no significant difference in total healthcare costs (mean difference: $146, p=0.228) between OM and TM patients. OM patients had significantly higher pharmacy costs (mean difference: $313, p<0.001) and significantly lower medical costs (mean difference: $460, p<0.001) compared to TM patients.

Conclusions: Patients stabilized on an OM LAI had lower frequency of HRU and lower medical costs, which offset the higher pharmacy costs compared to patients stabilized on a TM LAI.

For more information about Analysis Group's HEOR practice, please contact:

Howard G. Birnbaum Ph.D., economics, Harvard University
617 425 8108 | howard.birnbaum@analysisgroup.com

Mei Sheng Duh Sc.D., pharmacoepidemiology, Harvard School of Public Health
617 425 8131 | mei.duh@analysisgroup.com

Patrick Lefebvre M.A., economics, Université Laval
514-394-4471 | patrick.lefebvre@analysisgroup.com

Alan G. White Ph.D., economics, University of British Columbia
617-425-8217 | alan.white@analysisgroup.com

Eric Q. Wu Ph.D., pharmacoeconomics and policy, University of Southern California
617 425 8254 | eric.wu@analysisgroup.com

For bios and further information, please visit www.analysisgroup.com

About Analysis Group's HEOR Practice

Founded in 1981, Analysis Group is one of the largest private economics consulting firms, with more than 700 professionals across 11 offices. Analysis Group's health care experts apply analytical expertise to health economics and outcomes research, clinical research, market access and commercial strategy, and health care policy engagements, as well as drug-safety related engagements in epidemiology. Analysis Group's internal experts, together with its network of affiliated experts from academia, industry, and government, provide our clients with exceptional depth of expertise and end-to-end consulting services globally.

ANALYSIS GROUP
ECONOMIC, FINANCIAL and STRATEGY CONSULTANTS

www.analysisgroup.com

Boston   Chicago   Dallas   Denver   Los Angeles   Menlo Park   New York   San Francisco   Washington, D.C.   Beijing   Montreal