providers in better understanding the needs of the caregivers, thereby, ensuring optimal outcomes for their patient.

PND42

MEASUREMENT OF HEALTH RELATED QUALITY OF LIFE USING EQ-5D IN PATIENTS WITH STROKE IN INDIA

Marfatia S, Munshi S, Patel M

pharmEDGE, Mumbai, India

OBJECTIVES: To assess the impact of stroke on patient's health related quality of life (HRQOL) using EQ-5D. **METHODS:** A convenient sample of 300 patients were recruited from six medical centers across three cities (Mumbai, Bangalore, and Delhi) in India. Patients older than 18 years diagnosed with stroke between June 2011 to June 2012 who were receiving therapy for stroke were included in the study. Data on patients' socio-demographics, diagnosis, relevant clinical complications, were collected using a paper-based case report form. Patients were asked to rate their overall health using the EQ-VAS scale and data on the five dimensions of quality of life was collected using the EQ-5D questionnaire that was administered by trained interviewers. Translated and validated versions of the EQ-5D and EQ-VAS scales for five Indian languages were used in the study. **RESULTS:** The mean age of patients in the study was 58 (SD 14.45) and majority (70%) of the patients were males. Of the 300 patients, 60% had ischemic stroke. The mean EQ-VAS score was 66 (SD 23.09). Age and socioeconomic status were significant predictors ($p<0.05$, $p<0.001$ respectively) of EQ-VAS score. However, these variables were negatively correlated with the EQ-VAS score indicating that patients who were older and from an upper socioeconomic strata rated their overall health to be lower. Additionally, the frequency of problems was higher among older patients on mobility and usual care domains of the EQ-5D scale than younger patients. **CONCLUSIONS:** These findings suggest the value of measuring health status among stroke patients in addition to assessing clinical symptoms as it enables comprehensive evaluation of a patient's health condition.

PND43

IMPACT OF VELAGLUCERASE ALFA ON QUALITY OF LIFE OF ADULT PATIENTS WITH TYPE-I GAUCHER DISEASE

Elstein D[1], Enciu CA[2], Fontaine L[2], Piwko C[3]

[1]*Shaare-Zedek Medical Centre, Jerusalem, Israel,* [2]*Shire HGT (Canada) Inc., Toronto, ON, Canada,* [3]*PIVINA Consulting Inc., Mississauga, ON, Canada*

OBJECTIVES: Type-1 Gaucher Disease (GD1) causes anemia, thrombocytopenia, hepatosplenomegaly, and skeletal pathology. Patients' health-related quality of life (HRQoL) has been shown to be significantly diminished relative to the U.S. general population. Velaglucerase alfa is an enzyme replacement therapy (ERT) with the most comprehensive clinical trial registration program for an approved ERT in GD1 and has been reported as an effective and well-tolerated therapy. **METHODS:** The change in HRQoL, measured using the Short-Form-36 version 2 (SF-36 v2) in adults at baseline and 1 year in a randomized, double-blinded, parallel-group, two-dose Phase III clinical trial of velaglucerase alfa, was designated a priori a non-key secondary endpoint. **RESULTS:** The mean baseline (SD) was 42.3 (8.00) and 46.0 (5.90) for the norm-based Physical Component Summary (PCS) and 41.7 (12.6) and 48.1 (10.7) for the norm-based Mental Component Summary (MCS), respectively, for the 45 U/kg (N=8) and 60 U/kg (N=7) treatment arms; the mean U.S. normal population score=50; 1 SD=10). Fourteen adult GD1 patients had completed both a baseline and 1 year SF-36 HRQoL assessment (N=8 receiving 45 U/kg; N=6 receiving 60 U/kg). The respective mean change at 1 year [95% CI] for the PCS and MCS was 4.2 [-4.6, 13.0] and 4.1 [1.0, 7.2] at the low dose and 1.7 [-3.9, 7.2] and 1.2 [-7.9, 10.3] at the high dose. Non-significant increases were observed in nearly all of the 8 individual SF-36 v2 health domains compared to baseline, however, the mean increases, as well as the mean increases in the norm-based PCS and MCS summary measures, were aligned with the expected achievements of therapeutic goals for HRQoL. **CONCLUSIONS:** In addition to clinical efficacy and safety, velaglucerase alfa showed clinically important increases in HRQoL in adults as measured by the SF-36 v2 within 1 year, however, further studies are needed.

NEUROLOGICAL DISORDERS – Health Care Use & Policy Studies

PND44

CLINICAL AND ECONOMIC EVIDENCE THRESHOLDS FOR ORPHAN DRUGS: ARE REQUIREMENTS FOR FAVORABLE HEALTH TECHNOLOGY ASSESSMENT AND REIMBURSEMENT ON THE RISE?

Shih AY[1], Duong A[1], Tao C[2], Ransom JF[1], Spinner DS[3], White C[2], Doyle JJ[4], Faulkner EC[3]

[1]*Quintiles Consulting, Hawthorne, NY, USA,* [2]*Quintiles Consulting, Cambridge, MA, USA,* [3]*Quintiles Consulting, Durham, NC, USA,* [4]*Quintiles, Hawthorne, NY, USA*

OBJECTIVES: Orphan drugs are therapies for low prevalence or neglected diseases with high unmet need. Many drugs have received orphan status globally, and benefited from 5-10 year marketing exclusivity; reduced stakeholder evidence requirements with unmet need weighing heavily into health technology assessment (HTA) and reimbursement decision-making; and largely uncontested pricing. Given tightening health system budgets, the proliferation of high priced orphan drugs in the United States (US) and Europe (EU) have caused these therapies to come under increased scrutiny from HTA agencies and payers to demonstrate value. To gain insight into evolving market access requirements, we conducted a multimarket review of orphan drug HTAs and reimbursement decisions comparing them to top selling non-orphan drugs in the US and EU. **METHODS:** Global HTAs and coverage decisions for orphan and blockbuster drugs were characterized and compared based on health economic, clinical and other value-based requirements. An analysis of orphan drug pricing was also conducted where pricing was available. **RESULTS:** More than 20 orphan drug HTAs were identified, including those for genetic and central nervous system disorders, cancer and cardiovascular disease. HTA agencies and payers scrutinized the robustness of clinical evidence and lack of comparator data. Cost-effectiveness was also often evaluated, though with relaxed ICER thresholds compared to non-orphan drugs. Failure of an orphan drug to meet historically accepted evidentiary thresholds have led some evaluators to grant conditional reimbursement, with the proviso that further supportive evidence must be provided, although outright reimbursement denial has become increasingly common. **CONCLUSIONS:** Once considered "reimbursement-safe" for drug developers, to achieve unrestricted reimbursement from payers orphan drugs now face higher evidentiary hurdles that are increasingly similar to non-orphan drugs. New pharmaceuticals intent on achieving optimal market access as an orphan drug must factor the evolving requirements for demonstrating value into clinical and health economic evidence generation plans.

PND45

COMPARING ADHERENCE RATES TO DISEASE MODIFYING THERAPIES FOR MULTIPLE SCLEROSIS IN COMMERCIAL ENROLLEES AND PART D BENEFICIARIES

Carroll CA

Teva Pharmaceuticals, Kansas City, MO, USA

OBJECTIVES: To compare adherence rates, defined as the Medication Possession Ratio (MPR), for patients using alternative disease modifying therapies (DMTs) to treat multiple sclerosis (MS) that are enrolled in either a commercial insurance plan or through the Part D Medicare beneficiary program. **METHODS:** Study patients had >1 pharmacy DMT claim [interferon, glatiramer acetate (GA), fingolimod or natalizumab] and were enrolled in either a commercial health plan or Part D program between the dates of January 2010 and October 2012 for patients using interferon, GA and natalizumab and between the dates of October 2010 and October 2012 for patients using fingolimod. Mean monthly MPR rates were calculated as the percentage of time the patient had access to a specific medication. **RESULTS:** The MPR for 243,330 and 197,784 commercial and Part D beneficiaries, respectively, were calculated across the study period. Adherence rates for commercial enrollees were similar across disease modifying therapies ranging from 75.4% for a once daily oral therapy to 79% for natalizumab. However, adherence in Medicare Part D beneficiaries was consistently lower than patients enrolled in commercial plans ranging from a reduction in MPR of 0.27% for GA to 7.4% in patients taking once daily oral fingolimod treatment. **CONCLUSIONS:** Medicare Part D beneficiaries had lower rates of adherence compared to MS patient enrolled in commercial plans which could present a unique opportunity for medication management programs offered either through pharmaceutical companies, health plans or specialty pharmacies.

PND46

EFFECT OF NEUROLOGIST AMBULATORY VISITS ON USE OF DISEASE-SPECIFIC PHARMACOTHERAPIES FOR CHRONIC NEUROLOGICAL CONDITIONS

Ney JP[1], Watanabe JH[2], van der Goes DN[3]

[1]*University of Washington, Seattle, WA, USA,* [2]*Western University College of Pharmacy, Pomona, CA, USA,* [3]*University of New Mexico, Albuquerque, NM, USA*

OBJECTIVES: Neurologists care for chronic neurological illnesses through the use of disease-specific pharmacotherapies, but any licensed medical provider can prescribe these medications. Therefore, we evaluate effect of neurologist care on usage of disease-specific pharmacotherapies for chronic neurological diseases. **METHODS:** Survey respondents from the 2002-2010 Medical Expenditure Panel Survey(MEPS), an annual representative dataset of the civilian non-institutionalized US population, were identified for multiple sclerosis, parkinsonism, epilepsy, and dementia. Data collection included prescription classes and sub classes (from Cerner Multum) for determining immuno-modulatory, antiparkinsonian, anticonvulsant, and antidementia medications, identified neurologist ambulatory visits, age, illness duration, race, income, gender, education, comorbidities, region and year of care. Survey-weighted multivariate logistic and ordinary least squares regressions were performed on the outcomes of disease specific prescriptions and costs for the calendar year. Subgroup analyses were performed for each index condition. **RESULTS:** A total of 4948 MEPS respondents (weighted sample: 5.1 million US citizens, 95% CI 4.8m-5.4m) reported one or more index conditions. Only 27% reported a neurologist ambulatory care visit. Neurologist visits were associated with illness duration, high school graduate education, insurance coverage, and income. For the group seeing a neurologist, the adjusted likelihood of a patient with one of the four identified chronic neurological diseases receiving a disease-specific medication was substantially greater (OR 2.7, p-value <0.001) and adjusted annual expenditures for these medications was a mean of $1780 more (p-value <0.001); these estimates varied but were significant for all disease subgroups. **CONCLUSIONS:** Patients are much more likely to receive disease-specific pharmacotherapies for chronic neurological conditions when a neurologist is involved with their care. Policy makers and pharmaceutical companies should consider specialist physician involvement in care as a factor in determining the impact of new disease-specific medications.

PND47

HOW CAN WE USE DISCRETE EVENT SIMULATION TO IMPROVE ACCESS TO HEALTH CARE: AN ASSESSMENT OF INCREASING ACCESS TO CARE FOR CHILDREN WITH MEDICALLY REFRACTORY EPILEPSY

Lim ME[1], Bowen JM[2], Snead III OC[3], Worster A[1], Goeree R[2], Tarride JE[1]

[1]*McMaster University, Hamilton, ON, Canada,* [2]*PATH Research Institute, McMaster University, Hamilton, ON, Canada,* [3]*The Hospital for Sick Children in Toronto, Toronto, ON, Canada*