this study demonstrate, for the first time, the high resource utilization and financial burden experienced by sIBM patients in the USA. Further data collection of this type is needed to better understand the true economic burden of sIBM not only in US but globally.

NEUROLOGICAL DISORDERS – Patient-Reported Outcomes & Patient Preference Studies

PND41 DESCRIPTION OF PROPHYLACTIC DRUG UTILIZATION PATTERNS IN MIGRAINE PATIENTS

Sierra A1, Molley D1, DeSai P2, Enloe CJ, Kinson NY1, Birmbaum HG1, Corey-Lisle PK1
Sakra S2

1Analysis Group, Inc., Boston, MA, USA, 2Amgen Inc., Thousand Oaks, CA, USA, 1EMD Serono, Inc., Paramus, NJ, USA

OBJECTIVES: Describe medication utilization patterns of migraine prophylactics in commercially insured patients. METHODS: Adult migraineurs (ICD-9 code 346.4X) newly initiating migraine prophylactics (no claims for 12 months before first index prophylactic prescription) between January 2007 and March 2013 were identified from the OptumInsight employer claims database and followed for 6 months. Migraine prophylactics included antiepileptics (topiramate, divalproex, valproic acid), beta-blockers (propranolol, timolol), antidepressants (amitriptyline and onabotulinumtoxin A). Continuous enrollment was required for 12 months pre-index and 6 months post-index. To increase the specificity of migraine prophylactics, patients with prior diagnoses for conditions for which their prescribed prophylactics were also indicated (e.g., depression, paroxysmal headache, epilepsy, hyperhidrosis, migraines) were excluded. Outcomes of interest were medication adherence (medication possession ratio [MPR]), discontinuation (>30 day gap between prescriptions), and switching patterns. Continuous treatment of initial prophylactic was described using Kaplan-Meier curves. RESULTS: 19,881 patients initiated prophylactic treatment with 12,136 (61%), 3,037 (15%), 4,163 (21%), and 5,494 (28%) patients initiating antiepileptics, beta-blockers, amitriptyline, and onabotulinumtoxin A, respectively. Mean (SD) MPR for any prophylactic was 0.49 (0.31) (0.34 (0.27)–valproic acid to 0.67 (0.22)–onabotulinumtoxin A) with a mean (SD) of 89.2 (56.7) days on treatment over 6 months. Discontinuation rates were high ranging from 74% (topiramate and onabotulinumtoxin A) to 90% (valproic acid). Switching rates ranged from 6% (topiramate) to 20% (valproic acid). Between 46% (topiramate) and 68% (timolol) patients discontinued treatment after the first prescription, and median days to discontinuation of initial treatment ranged from 30 (valproic acid, divalproex, timolol, amitriptyline) to 84 days (onabotulinumtoxin A). CONCLUSIONS: Adherence to migraine prophylactic medications was poor with about 50% of patients discontinuing after their first prescription and over 75% discontinuing within 6 months. The large proportion of patients discontinuing after first prescription is needed for reasons on discontinuation and better tolerated therapies.

PND42 A REVIEW OF METHODOLOGIES USED TO ASSESS ADHERENCE TO DISEASE MODIFYING THERAPIES AMONG PATIENTS WITH MULTIPLE SCLEROSIS

Thaile A1, Chinnam C1, Makwana T2, Brown A3
1The University of Texas at Austin, Austin, TX, USA

OBJECTIVES: To review the methods currently used to measure adherence to oral and injectable disease modifying therapies (DMTs) in multiple sclerosis (MS) patients and review how these methods are described in systematic literature. METHODS: MESH (Medical Subject Headings), CINAHL, PsycINFO, and Cochran Library to identify articles assessing adherence to DMTs. The publication time frame was from January 2004 to November 2014. RESULTS: 126 articles were included if they focused on at least one U.S. FDA-approved disease-modifying DMT, assessed DMT adherence either as a primary or secondary outcome, reported DMT adherence rate(s), and included details of the method(s) used to calculate adherence level or proportion of adherers/non-adherers. RESULTS: A total of 46 articles were identified. A total of 56 DMTs were assessed for adherence. CONCLUSIONS: It is our hope that this systematic review will help facilitate future research on MS treatment adherence.

PND43 ADHERENCE AND PERSISTENCE TO ANTI-EPILEPTIC DRUGS AMONG U.S. VETERANS DIAGNOSED WITH EPILEPSY

Velez FF1, 2, Baser O2, Xie L1
1Sanofi Pharmaceuticals Inc, Marlborough, MA, USA, 2StatinMED Research, The University of Michigan, MEF University, Ann Arbor, MI, USA

OBJECTIVES: To evaluate patient adherence and persistence to anti-epileptic drug (AED) treatment in U.S. veterans. METHODS: Adult patients with a diagnosis claims (ICD-9-CM:345) or one epilepsy diagnosis claim and one claim for another condition (ICD-9-CM: 780.39) were selected from the U.S. Veterans Health Administration database (01OCT2000-03JUL2013). Patients were required to have ≥1 AED prescription post-epilepsy diagnosis, and the first AED prescription claim date was designated as the index date. Continuous health plan enrollment around the index date and post-index were required. Patients assigned to four monotherapy AED cohorts based on drug class: sodium channel blockers (SCs), gamma-aminobutyric acid analogs (GABAAs), synaptic vesicle protein 2A binding (SV2) and multiple mechanisms (MMs). Adherence was assessed using the proportion of days covered (PDC) and adherence gap (AG) with discontinuation with an allowable treatment gap of 45 days without the index AED. Logistic and Cox proportional hazards models were used to compare the results among the four AED cohorts. RESULTS: Patients’ index date was 12/11/2000. On average, 32% of patients were prescribed with an SC, 23% a GABAAs, 15% a SV2, and 30% a MM. Patients treated with GABAAs (OR=0.44, p<0.001) and MMs (OR=0.63, p<0.001) were significantly less likely to adhere to their medications (PDC <80%) than those treated with SCs and SV2. Moreover, patients prescribed with SCs had a 5% confidence interval [Cl]=1.59-1.90 and MMs (HR=1.18, 95% CI=1.07-1.29) were more likely to discontinue treatment during the follow-up period compared to those in the SC cohort. CONCLUSIONS: Patients treated with Sodium channel blockers were less likely to discontinue treatment than those treated with SCs and SV2.

PND44 MEASURING ADHERENCE AND OUTCOME IN TREATMENT OF MULTIPLE SCLEROSIS IN THE GEISINGER CLINIC

Velez FF1, 2,3, Baser O2, Xie L1
1Geisinger Health System, Danville, PA, USA, 2Liberty University, Lynchburg, VA, USA, 3Sutter Health, Research, Development & Dissemination, Walnut Creek, CA, USA, 4University of Pittsburgh, PA, USA

OBJECTIVES: To review the methods currently used to measure adherence to DMTs. The publication time frame was from January 2004 to November 2014. METHODS: MESH (Medical Subject Headings), CINAHL, PsychINFO, and Cochrane Library to identify articles assessing adherence to DMTs. The publication time frame was from January 2004 to November 2014. RESULTS: 126 articles were included if they focused on at least one U.S. FDA-approved disease-modifying DMT, assessed DMT adherence either as a primary or secondary outcome, reported DMT adherence rate(s), and included details of the method(s) used to calculate adherence level or proportion of adherers/non-adherers. RESULTS: A total of 46 articles were identified. A total of 56 DMTs were assessed for adherence. CONCLUSIONS: It is our hope that this systematic review will help facilitate future research on MS treatment adherence.

PND45 WHAT ARE PATIENTS WILLING TO PAY FOR WHOLE GENOME SEQUENCING INFORMATION?

Marshall DA1, Gonzalez JM2, Johnson FR3, Pugh A4, MacDonald KV4, Douglas MP5
1Allerta Bone and Joint Health Institute, Calgary, AB, Canada, 2ZTH Health Solutions, Research Triangle Park, NC, USA, 3Duke University, Durham, NC, USA, 4University of Calgary, Calgary, AB, Canada, 5University of California 8¢¢ San Francisco, San Francisco, CA, USA

OBJECTIVES: Whole genome sequencing (WGS) can be used to predict future disease risk or inform treatment. Current guidelines suggest only reporting variants that are clinically actionable. Reporting incidental or non-actionable findings could generate anxiety and unnecessary medical tests, but patients could miss valuable information if not reported. Over-treatment may occur by acting on findings prematurely, potentially causing harm and unnecessary resource use. We measure the value of WGS information using contingent valuation methods. METHODS: An online pilot survey (n=26 adults from US general population) was used to evaluate willingness to pay for a basic WGS report (recommended by guidelines), and genetic information excluded from the basic report (non-actionable findings) to inform a national survey. RESULTS: Most respondents (n=11) were willing to pay more than $1000 for the basic report. Most respondents (n=17, 65%) were not willing to pay anything for the basic report, and no respondent was willing to pay more than $1000 for the basic report. Most respondents (n=17, 65%) were not willing to pay anything for non-actionable genetic information, and only one person reported being willing to pay more than $400 for it. CONCLUSIONS: A large number of participants perceived that genetic information can be harmful, as shown by respondents’ lack of interest in this information even if it were free. Our findings also suggest that respondents were willing to pay more for actionable genetic information than for non-actionable findings.
scored on a 0 (no difficulty) to 10 (unable to do) numerical rating scale. sFA items are aligned with the functional impairments of sIBM described in the literature and expert review and identified as relevant and important to sIBM patients. The draft conceptual framework includes items related to upper extremity, lower extremity, general function and swallowing. Cognitive testing of paper and eFAQ versions support the functional tool in a b-line. eFAQ and properties of sIBM: The sFAQ is the first content valid tool developed specifically for use in the functional assessment of treatment benefit in sIBM patients aligned with the FDA PRO Guidance. Psychometric evaluation is underway.

PND49

UNDERSTANDING THE SUITABILITY OF CYSTIC FIBROSIS–SPECIFIC CLINICAL OUTCOMES ASSESSMENTS FOR CLINICAL TRIALS AND TO SUPPORT MEDICAL PRODUCT LABELING

Willigts TP, Trigge A, Meyers S, Kitchen H, Humphrey L, Blankenburg M

Abstract

OBJECTIVES: To identify and review the suitability of cystic fibrosis (CF)-specific clinical outcomes assessments (COAs) for clinical trial assessment of novel CF therapies and to support product labeling. METHODS: CF-specific COAs were identified and appraised for suitability using multiple criteria terms and via clinical trials. COA utilization in CF clinical trials and previous success in supporting product labeling was also explored. In line with best-practice, conceptual coverage of identified COAs was assessed by mapping items to a patient-centered conceptual model of CF symptoms and impacts. COAs with the most comprehensive conceptual coverage were further evaluated for content validity, psychometric properties and feasibility of use. RESULTS: Nine CF-specific COAs were identified, Cystic Fibrosis Questionnaire-Revised (CFQ-R) [pediatric 6-11/12-13 years, 14+ years and parent-report 6-13 years] and Cystic Fibrosis Respiratory Symptom Diary (CFRSD) were selected for in-depth review. CFQ-R 14+ provided the most comprehensive conceptual coverage of assessed symptom dimensions and 50% of COA concepts. Pediatric and parent-reported versions of CFQ-R assessed fewer concepts. All versions of CFQ-R have acceptable psychometric properties and are linguistically validated in >10 languages. CFQ-R has also supported product labeling claims for respiratory symptoms. Limitations include the acceptability of a 2-week recall period and inconsistencies in concepts measured across pediatric and adult versions. CFSQ provides a comprehensive assessment of 83% of acute respiratory symptoms in addition to assessing 52% of CF-related impacts. For FADA Drug Development Tool qualification by FDA. The respiratory symptom score has excellent psychometric properties; impact items are yet to be validated. CONCLUSIONS: Although 9 CF-specific COAs were identified in this study, only the CFQ-R measures and CFRSD appear potentially suitable for assessment of CF-related symptoms and impacts in CF clinical trials. Further data relating to content validity of CFQ-R may be required to support future labeling approvals.

PND50

THE PARKINSON’S DISEASE QUESTIONNAIRE (PDQ-39) - EVALUATING THE PSYCHOMETRIC PROPERTIES OF AN ELECTRONIC VERSION

Moriely DL, Jenkinson C1, Dummett SJ, Kelly L1, Churchman DR2, Dawson J1

OBJECTIVES: The 39-item Parkinson’s Disease Questionnaire (PDQ-39) is the most thoroughly validated and extensively used self-report measure for the assessment of health-related quality of life in people with Parkinson’s (PwP). The measure has been shown to possess sound psychometric properties and its use is widely recommended as a preferred outcome measure for FADA drug development. The purpose of this study was to systematically review the psychometric properties of the paper-based version of the PDQ-39 as well as assess the suitability of the questionnaire for the FADA Drug Development Tool qualification by FDA. The PDQ-39 is a Likert-type scale ranging from 1 (no impact) to 5 (extreme impact). The measure comprises 6 domains: mobility, activities of daily living, communication, emotional well-being, and social support. The PDQ-39 has been extensively used in clinical trials and can be administered in less than 15 minutes. The PDQ-39 has been shown to be a valid and reliable measure of health-related quality of life in people with Parkinson’s. The measure has been shown to possess sound psychometric properties and its use is widely recommended as a preferred outcome measure. The PDQ-39 is a Likert-type scale ranging from 1 (no impact) to 5 (extreme impact). The measure comprises 6 domains: mobility, activities of daily living, communication, emotional well-being, and social support. The PDQ-39 has been extensively used in clinical trials and can be administered in less than 15 minutes. The PDQ-39 has been shown to be a valid and reliable measure of health-related quality of life in people with Parkinson’s. The measure has been shown to possess sound psychometric properties and its use is widely recommended as a preferred outcome measure. The PDQ-39 is a Likert-type scale ranging from 1 (no impact) to 5 (extreme impact). The measure comprises 6 domains: mobility, activities of daily living, communication, emotional well-being, and social support. The PDQ-39 has been extensively used in clinical trials and can be administered in less than 15 minutes. The PDQ-39 has been shown to be a valid and reliable measure of health-related quality of life in people with Parkinson’s. The measure has been shown to possess sound psychometric properties and its use is widely recommended as a preferred outcome measure.

PND62

SIX DIFFERENCES IN OVER-THE-COUNTER SLEEP AID USE IN OLDER ADULTS

Gross HJ1, O’Neill G2, Toscani M, Chapman J1

OBJECTIVES: Over-the-counter (OTC) sleep aids are widely self-administered and are generally safe, but their primary ingredients (diphenhydramine and doxylamine [DOXY]) may produce adverse effects in older adults. A recent review of the FDA’s Over-the-counter (OTC) sleep aid website from the 2013 US National Health and Wellness Survey (USNHS) is a cross-sectional, internet-based, IRB-approved annual survey of adults (N=75,000). Stratified sampling was used to represent the demographic make-up of the general population in age, sex, and ethnicity. Weighted samples were available using age, sex, ethnicity, and general population proportion. The unknown regular singular sleeplessness and sleepiness were included in the analysis. Comparisons between the with chi-square tests for categoric variables and t-tests for continuous variables. RESULTS: Of the project’s 41.3 M (n=16,500) adults ≥65, 15% (3.8 M age 65-74, 26 M age 75+) reported...