VALIDATION OF THE CHILDHOOD HEALTH ASSESSMENT QUESTIONNAIRE (CHAQ) IN HUNTER SYNDROME

Tran KT1, Gold KF1, Stephens JM1, Kimura A2, Muenzer J1, Singh G1

1Abt Associates Inc, Bethesda, MD, USA; 2Transkaryotic Therapies Inc, Cambridge, MA, USA; 3University of North Carolina at Chapel Hill, Chapel Hill, NC, USA; 4Stanford University, Palo Alto, CA, USA

OBJECTIVES: Mucopolysaccharidosis II (MPS II; Hunter syndrome [HS]), a rare genetic, lysosomal storage disease. METHODS: The survey item pool was tested in MPS II patients and their families attending the 2003 Annual MPS Society Family Conference. Data were obtained via structured feedback forms completed during face-to-face interviews. Inter-item correlations within each domain were tested for internal reliability. Results were used to modify items to create a finalized instrument, the HS-FOCUS. RESULTS: Two versions of the HS-FOCUS were created. The parent-reported form included 59 items and the patient-reported form included 58 items, in the domains of standing/walking, grip/reach, sleeping, schooling, activities, eating/appetite, satisfaction and botheredness with functional status, and treatment satisfaction. Sixteen parents and 2 adult MPS II patients completed the questionnaire and provided feedback on relevance of questions, understandability, and feasibility of use. Domains of grip/reach, schooling, activities, satisfaction, and botheredness with physical function achieved a Cronbach’s alpha of at least 0.84. Based on these analyses for construct validity along with written and interview feedback, 16 items were revised for clarity and specificity, 6 were removed, and 9 were added. CONCLUSIONS: Preliminary results suggest that the HS-FOCUS may be useful for assessing functional health in MPS II. Inasmuch as existing instruments do not adequately assess functional outcomes in patients with MPS II, this preliminary version of the HS-FOCUS may offer clinicians and researchers an opportunity to do so. Future studies should validate the instrument.

DEVELOPMENT AND PILOT TESTING OF THE HUNTER SYNDROME-FUNCTIONAL OUTCOMES FOR CLINICAL UNDERSTANDING SCALE (HS-FOCUS): AN INSTRUMENT TO ASSESS FUNCTIONAL HEALTH IN HUNTER SYNDROME

Tran KT1, Stephens JM1, Gold KF1, Kimura A2, Pashos CL3, Muenzer J4

1Abt Associates Inc, Bethesda, MD, USA; 2Transkaryotic Therapies Inc, Cambridge, MA, USA; 3Abt Associates Inc, Cambridge, MA, USA; 4University of North Carolina at Chapel Hill, Chapel Hill, NC, USA

OBJECTIVES: To develop a new instrument, the Hunter Syndrome-Functional Outcomes for Clinical Understanding Scale (HS-FOCUS), a new instrument for assessing functional status in children and adolescents with Mucopolysaccharidosis II (MPS II; Hunter Syndrome [HS]), one of a group of rare genetic, lysosomal storage diseases. METHODS: Following IRB approval, participants were recruited from the 2003 Annual MPS Family Conference and an ongoing MPS II Phase IIIb clinical trial. Eligible participants were individuals with MPS II at least 12 years old or a parent of a child of any age with MPS II. The instrument was administered twice over a 3-week period. Face validity, internal reliability, domain intercorrelation, parent-child correlations, and test-retest reliability were assessed for each of six domains (standing/walking, grip/reach, sleeping, schooling/work, activities, and breathing) and for the overall disability score. RESULTS: Eleven patients with MPS II and 27 parent caregivers of patients with MPS II completed the HS-FOCUS. Face validity was confirmed through interviews with expert clinicians, patients with MPS II, and their families. The instrument showed very good overall internal reliability (Cronbach’s alpha = 0.93 [parents] and 0.83 [patients]). The HS-FOCUS showed good reproducibility (r = 0.85, p < 0.0001 [parents] and 0.71, p = 0.031 [patients]) for overall function in test-retest analyses, although sleeping and breathing domains had weaker correlations. Intercorrelation coefficients for each domain with the overall functional disability score were strong (range r = 0.69 to 0.89). Weak correlations were reported between the nine parent-child pairs, which is commonly accepted as a challenge in survey research of children and adolescents. CONCLUSIONS: Findings of this validation study suggest that the HS-FOCUS may effectively capture disability and functional status in individuals with MPS II. Additional assessment of sen-
sitivity and construct validity are warranted, and could be conducted as part of ongoing clinical trials.

NEUROLOGICAL/GENETIC DISORDERS (Migraine, Alzheimer’s, Parkinson’s, MS, Epilepsy, Brain Injury, Hunter Syndrome, Insomnia)

NEUROLOGICAL/GENETIC DISORDERS (Migraine, Alzheimer’s, Parkinson’s, MS, Epilepsy, Brain Injury, Hunter Syndrome, Insomnia)—Health Policy Studies

PNL20
UTILIZATION OF IMMUNOMODULATORY DRUG THERAPIES IN MULTIPLE SCLEROSIS (MS) IN NOVA SCOTIA, CANADA 1998–2003
Skepris IS, Hicks V, Lummis H, Brown M
1Dalhousie University, Halifax, NS, Canada; 2Capital District Health Authority, Halifax, NS, Canada

OBJECTIVE: Immunomodulatory drugs have provided hope to patients with MS. The Nova Scotia provincial health ministry funds these drugs for patients who are seen by the Dalhousie Multiple Sclerosis Research Unit (DMSRU), meet predefined criteria and attend an education clinic. This study examined the utilization of these drugs under the provincial program.

METHODS: Data from the DMSRU and pharmacy dispensing records was accessed from July 1, 1998 to September 16, 2003. 2035 patients attended the clinic of whom 1819 were diagnosed with MS. The Nova Scotia provincial health ministry funded these drugs for patients who are seen by the Dalhousie MS Research Unit (DMSRU), meet predefined criteria and attend an education clinic. This study examined the utilization of these drugs under the provincial program.

RESULTS: A total of 433 patients (326 F) received immunomodulatory drug therapy. Fifty-nine percent of patients were between 35 and 49 years of age and 36% were classified as relapsing remitting MS. Funded patients increased from 98 in 1998 to 365 in 2002. In 2001 the median drug cost/patient was CDN $15,508. The median number of prescriptions/year was 12.0 (mean 10.7 ± 3.3). 84% of patients received a prescription in 2 fiscal years, while 17% received a prescription in all 5. In 2001, 60% of patients had a maximum EDSS score of 5 or less. Patients receiving immunomodulatory drugs were less likely to have EDSS scores over 6 compared to those not receiving these drugs. The total expenditure for MS drugs was CDN $4.96 million in 2001/2 with Rebif® accounting for $1.6 million (CDN). CONCLUSION: Expenditures grew rapidly for the program (from $0.83 million in 1998 to $4.96 million in 2001). Most patients were compliant receiving 12 prescriptions/year. Further work is ongoing to compare patient outcomes and health care costs in those patients receiving therapy to those who are not.

PNL21
INSOMNIA IN A NATIONAL AMBULATORY CARE SETTING 2001
Stafke D, Morlock R
1University of Michigan/ Pfizer; Ann Arbor, MI, USA; 2Pfizer, Inc, Ann Arbor, MI, USA

OBJECTIVES: The purpose of this study was to estimate the number of physician visits for a primary complaint of insomnia and characterize patients with a primary complaint of insomnia, diagnosis of insomnia and patients utilizing sleep medications.

METHODS: Data was obtained from the 2001 version of the National Ambulatory Medical Care Survey. Descriptive analyses were utilized to examine individuals with a primary complaint of insomnia and/or diagnosed with insomnia. Patient level weights were utilized to derive national population estimates from a representative sample.

RESULTS: In 2001, there were 1.6 million patient visits for a primary complaint of insomnia. More females than males (87% vs. 17%), and more Caucasians than other races (64% vs. 36%) reported insomnia as their primary complaint. While only 22% of patients complaining of insomnia were diagnosed with a sleep disorder, a significant number (79%) were prescribed a medication, including Ambien or Sonata (26%) and Benadryl (10%). During the same year, 4.8 million patients were diagnosed with a sleep disorder. Patients were diagnosed by generalist (67%), Psychiatrist (3%) and other specialist (43%). Yet, only 9% of patients diagnosed with a sleep disorder had a primary complaint of insomnia upon visiting their physician. Medications were utilized by 76% of these patients including Ambien or Sonata (15%) and Benadryl (3%). Only 15% of patients using Ambien or Sonata and only 4% of patients taking Benadryl were diagnosed with a sleep disorder. CONCLUSION: This work describes the characteristics of patients with a primary complaint of insomnia and their resultant diagnosis and pharmaceutical treatment. Additionally, we look at patients diagnosed with insomnia and describe the most common patient reported reason for their visit and their pharmaceutical treatment.

PNL22
THE SENSITIVITY OF COST-EFFECTIVENESS ESTIMATES IN MULTIPLE SCLEROSIS TO INTERNATIONAL DIFFERENCES IN NATURAL HISTORY: SWEDEN VERSUS NOVA SCOTIA, CANADA
Skegel CD, Brown MG, MacKinnon-Cameron D
Dalhousie University, Halifax, NS, Canada

OBJECTIVE: To investigate the sensitivity of cost-effectiveness (CE) estimates of drug treatment that delays disability progression in Multiple Sclerosis (MS) to international differences in the underlying natural history of the disease. METHODS: Simulation model Multiple Sclerosis PharmacoEvaluation Tool (MS-PEET) was developed to estimate the CE of drug treatment that delays disability progression in Multiple Sclerosis. MS-PEET was initially populated with Swedish data on the natural history of disability progression, measured by the cumulative probability of patients reaching three disease-specific severity endpoints (EDSS 3, 6, 10). Treatment effectiveness is modeled as a reduction in the probability of EDSS progression. This analysis compares CE estimates based on Nova Scotia natural history data with estimates based on Swedish data, holding all other variables constant. Nova Scotia natural history data is from the Dalhousie Multiple Sclerosis Research Unit (DMSRU). The DMSRU has up to 25 years of clinical follow-up for 2368 patients. RESULTS: Preliminary analysis of untreated patients in DMSRU data shows a less severe natural history course in Nova Scotia relative to Sweden. The reported cumulative probability of progressing to severe disability (EDSS ≥ 6) within 10 years of MS-onset is almost 60 percent less in Nova Scotia than in Sweden. CE estimates based on Nova Scotia natural history data are roughly 150 percent higher than estimates based on Swedish data. CONCLUSION: A less severe MS natural history course limits potential gains in terms of disability years avoided with treatment. Consequently estimates of cost-effectiveness are likely to be sensitive to differences in the underlying natural history. MS-