44mcg was chosen as comparator since it is the most widely prescribed disease modifying therapy for 1<sup>st</sup>-line treatment in HARRMS patients in Brazil. **METHODS:** We developed a Markov model with 20-year time horizon comparing natalizumab to IFNB1a 44mcg. Health states were based on EDSS and relapses (moderate or severe). Since there are no published data evaluating long-term course specifically in HARRMS, we assumed transition probabilities on EDSS states based on natural history studies on unselected RRMS patients, and relapse probabilities based on a post-hoc analysis of the pivotal natalizumab AFFIRM trial. This is a rather conservative approach, since disability progression may be slower in this proposed model then expected for patients with HARRMS and so the benefit from natalizumab could be underappreciated. In each monthly cycle, patients can discontinue treatment, remain stable, progress to higher EDSS state, experience Progressive Multifocal Leukoencephalopathy or die. Patients with EDSS score≥7.5 receive best supportive care. Resource use and costs were validated by an expert's panel and valued using Brazilian public official lists (DATASUS and BPS). Costs and outcomes were discounted (5%). Probabilistic sensitivity analyses (PSA) covered variability in efficacy and costs. RESULTS: Use of natalizumab was associated with slower EDSS progression and reduced relapse burden. Life years gained with natalizumab and IFNB1a 44mcg were 10.90 and 10.54, and costs were USD119,977 and USD132,446, respectively. In the base-case, natalizumab was dominant versus IFNB1a 44mcg. PSA has confirmed the consistency of results. CONCLUSIONS: For a patient with HARRMS, the model shows that natalizumab was dominant when compared to IFNB1a 44mcg in the Brazilian Public Healthcare System.

## PND13

# BURDEN OF MULTIPLE SCLEROSIS AND UNMET NEEDS IN BRAZIL: HEALTH CARE RESOURCE UTILIZATION

Silva NL<sup>1</sup>, Takemoto M<sup>2</sup>, Damasceno B<sup>3</sup>, Fragoso YD<sup>4</sup>, Finkelsztejn A<sup>5</sup>, Gomes M<sup>1</sup> <sup>1</sup>Novartis Biociências S.A., São Paulo, Brazil, <sup>2</sup>ANOVA - Knowledge Translation, Rio de Janeiro, Brazil, <sup>3</sup>UNICAMP - Hospital de Clínicas, Campinas, Brazil, <sup>4</sup>UNIMES - Universidade Metropolitana de Santos, Santos, Brazil, <sup>5</sup>Hospital de Clínicas de Porto Alegre, Porto Alegre, Brazil OBJECTIVES: To assess the health care resource utilization (HRU) of Brazilian multiple sclerosis (MS) patients. METHODS: This was a cross-sectional, multicenter study conducted in 8 Brazilian major MS treatment sites. HRU was evaluated as the percentage of patients self-reporting the consumption of resources. The main categories were: hospitalization, consultations, laboratory and imaging tests, disease modifying therapies (DMTs), co-medication, aidsand/or home modifications. Frequency and average consumption were annualized. RESULTS: The study enrolled 210 MS patients, mean age was 40.7 [standard deviation=11.5] years and 70.7% female. Patients with mild disability (according to self-reported Expanded Disability Status Scale [EDSS]) represented 40.4% of patients, 43.7% had moderate disability and 15.9% had severe disability. Hospitalization was reported by 23%, 33% and 15%  $\,$ of mild, moderate and severe MS patients, with average length of stay of: 7.53, 10.41, and 7.40 days, respectively. Most patients (>96%) had at least one neurologist consultation per year (average 4.94 visits/year in the total sample). Physical therapy was the most consumed non-medical consultation (mild: 11%; moderate: 38%; severe: 64%). Magnetic resonance imagingwas reported by 60%, 68%, and 55% of mild, moderate and severe patients. Patients using at least one DMT during the previous year were: 89%, 93%, and 61% of mild, moderate and severe MS patients, respectively. The most prescribed DMT was glatiramer acetate (38%, total sample). The most frequent co-medications were: anti-depressants, anti-spasticity, and analgesics. Home modification was reported by 19% and 45% of patients with moderate and severe disability, respectively. For ambulation, walking stick was used by 35% of moderate patients, while wheelchair was needed by 58% of severe patients. CONCLUSIONS: To our knowledge, this is the first Brazilian study investigating the HRU of MS patients. The findings can be useful to better understand MS patients' needs in terms of comprehensive care.

NEUROLOGICAL DISORDERS - Patient-Reported Outcomes & Patient Preference Studies

## PND14

## WHICH CYSTIC FIBROSIS INHALED ANTIBIOTIC MEDICINE FEATURES MATTER MOST TO ADULT PATIENTS AND PARENTS OF PEDIATRIC PATIENTS?

Mohamed AF<sup>1</sup>, Johnson FR<sup>1</sup>, Balp MM<sup>2</sup>, Calado F<sup>2</sup>

<sup>1</sup>RTI Health Solutions, Research Triangle Park, NC, USA, <sup>2</sup>Novartis Pharma AG, Basel, Switzerland OBJECTIVES: To quantify patient and parent preferences and adherence for different administration features of inhaled antibiotic medicines for cystic fibrosis (CF). METHODS: Adult patients with a self-reported physician diagnosis of CF and parents of pediatric CF patients (6 to 17 years) who had Pseudomonas aeruginosa in their lung culture at least twice in one year completed a web-enabled, discretechoice experiment survey in the United States. Respondents answered 5 treatment-choice questions with known statistical properties. Each question required evaluating a pair of hypothetical CF treatment profiles defined by device type (nebulizer, dry powder inhaler (DPI)), total daily administration and cleaning time, dosing frequency, dry cough side effect, and personal cost per cycle. Lung function measured as forced expiratory volume in one second (FEV1) was held constant between the hypothetical CF treatment profiles. Stated adherence questions followed two randomly selected treatment-choice questions. Random-parameters logit models were used to estimate preference weights for all feature levels and the mean relative importance of each feature for both samples. **RESULTS:** A total of 209 adult patients and 271 parents completed the survey. Mean age of adult patients was 32 (SD = 10) years and mean age of pediatric patients was 12 (SD = 3) years. Among all respondents, the average time spent taking inhaled antibiotic medicines was approximately 40 minutes. Relative importance estimates indicated that switching from a 30-minute nebulizer twice daily to a 10-minute DPI twice daily was 6.3 times more important for adult patients and 2.0 times more important for parents than an improvement in dry cough from moderate to mild. Stated adherence for adult and pediatric patients was 20-30% higher for DPIs versus nebulizers. CONCLUSIONS:

Treatments administered with more convenient devices such as DPIs and shorter administration times are associated with higher utility and higher stated adherence in adult and pediatric patients.

## PND15

BURDEN OF MULTIPLE SCLEROSIS AND UNMET NEEDS IN BRAZIL: MEASUREMENT OF FATIGUE USING MODIFIED FATIGUE IMPACT SCALE <u>Silva NL</u><sup>1</sup>, Takemoto M<sup>2</sup>, Damasceno B<sup>3</sup>, Fragoso YD<sup>4</sup>, Finkelsztejn A<sup>5</sup>, Gomes M<sup>1</sup> <sup>1</sup>Novartis Biociências S.A., São Paulo, Brazil, <sup>2</sup>ANOVA - Knowledge Translation, Rio de Janeiro, Brazil, <sup>3</sup>UNICAMP - Hospital de Clínicas, Campinas, Brazil, <sup>4</sup>UNIMES - Universidade Metropolitana de Santos, Santos, Brazil, <sup>5</sup>Hospital de Clínicas de Porto Alegre, Porto Alegre, Brazil OBJECTIVES: Fatigue is one of the most frequent symptoms in patients with multiple sclerosis (MS). This study aimed to examine the severity and impact of fatigue in MS Brazilian patients. METHODS: This was a cross-sectional, multicenter study conducted in 8 Brazilian major MS treatment sites. Fatigue was assessed using the Brazilian version of the Modified Fatigue Impact Scale (MFIS), which evaluates the impact of fatigue on 3 dimensions of patients' daily life: physical, cognitive and psychosocial. The patient scores 0 (lower impact) to 84 points (higher impact). The final score was classified according to the level of impact: absent (0-38), low (39-58), and high (>58). **RESULTS:** The study enrolled 210 MS patients, of which the mean age was 40.7 [standard deviation = 11.5] years and 70.7% were female. Patients with mild disability (according to self-reported Expanded Disability Status Scale [EDSS]) represented 40.4% of patients, 43.7% had moderate disability and 15.9% had severe disability. In the overall sample, the impact of fatigue was considered absent, low and high in 49%, 32% and 19% of patients, respectively. Any impact (both low and high summed) was reported by 33%, 63%, and 66% of patients with mild, moderate and severe disability, respectively. The mean MFIS total score for mild, moderate and severe patients was 29.3, 45.0, and 45.4 (38.6 in the total sample). The mean impact scores for each domain in the total sample were 20.0 (physical, range 0-36), 14.7 (cognitive, range 0-40), and 3.9 (psychosocial, range 0-8), meaning that fatigue has a proportionally higher impact in the physical than the cognitive or psychosocial domains. CONCLUSIONS: Our findings indicate that over 50% of MS Brazilian patients notice some adverse impact of fatigue in their daily lives, particularly related to the physical domain.

## PND16

BURDEN OF MULTIPLE SCLEROSIS AND UNMET NEEDS IN BRAZIL: MEASUREMENT OF HEALTH-RELATED QUALITY OF LIFE USING EQ-5D

<u>Silva NL</u><sup>1</sup>, Takemoto M<sup>2</sup>, Damasceno B<sup>3</sup>, Fragoso YD<sup>4</sup>, Finkelsztejn A<sup>5</sup>, Gomes M<sup>1</sup> <sup>1</sup>Novartis Biociências S.A., São Paulo, Brazil, <sup>2</sup>ANOVA - Knowledge Translation, Rio de Janeiro, Brazil, <sup>3</sup>UNICAMP - Hospital de Clínicas, Campinas, Brazil, <sup>4</sup>UNIMES - Universidade Metropolitana de Santos, Santos, Brazil, <sup>5</sup>Hospital de Clínicas de Porto Alegre, Porto Alegre, Brazil OBJECTIVES: To measure the health-related quality of life (HRQL) of multiple sclerosis (MS) patients and examine potential associations between HRQL and patients' characteristics. METHODS: This was a cross-sectional, multicenter study conducted in 8 Brazilian major MS treatment sites. HRQL was assessed using the Brazilian version of the EQ-5D and patients self-evaluated their HRQL and health status using five dimensions (mobility, self-care, usual activities, pain/discomfort and anxiety/depression) and a visual analog scale (VAS). The EQ-5D index was calculated based on the value set derived from the UK population, since the Brazilian value set is not available. RESULTS: The study enrolled 210 MS patients, of which the mean age (standard deviation [SD]) was 40.7 [11.5] years and 70.7% were female. Patients with mild disability (according to self-reported Expanded Disability Status Scale [EDSS]) represented 40.4% of patients, 43.7% had moderate disability and 15.9% had severe disability. Among the 5 assessed HRQL dimensions, the ones with higher frequency of self-reported severe limitations were usual activities and anxiety/depression (11.0% each). The least impaired dimension was self-care, with 63.0% of patients reporting absence of limitation. The mean [SD] VAS score was 71.6 [18.9]. The mean EQ-5D index for each level of EDSS (mild, moderate or severe disability) was 0.73 [0.21], 0.49 [0.30], and 0.30 [0.34], respectively. In the multivariate analysis, variables related to patients' characteristics were explored and the following were associated with the presence of any limitation in at least one dimension: older age, unemployment/retirement, relapses in the previous year, emergency department visits in the previous 6 months and lower educational level. **CONCLUSIONS:** MS adversely impact patients' HRQL, especially with disability progression and clinical features that can be linked to more severe disease

NEUROLOGICAL DISORDERS - Health Care Use & Policy Studies

#### PND17

## EVALUATION OF TREATMENT PATTERNS AND CLINICAL TRIALS PUBLISHED ON PATIENTS DIAGNOSED WITH INSOMNIA: A LITERATURE UPDATE Greene N<sup>1</sup>, Greene M<sup>2</sup>

<sup>1</sup>Massachusetts College of Pharmacy and Health Sciences, Boston, MA, USA, <sup>2</sup>Georgia State University, Bethel, MA, USA

**OBJECTIVES:** To conduct a systematic review of literature in peer-reviewed journals on real world treatment patterns and clinical trials on patients diagnosed with Insomnia. **METHODS:** A comprehensive literature search was performed using relevant search terms to identify articles published from 2000 to 2010 on the real world treatment patterns and clinical trials conducted on patients with Insomnia. Studies were identified through electronic Embase, Cochrane, Medline, and PubMed databases. Additional parameters were placed on the final search strategy to limit the retrieval to articles written in English, involving human subjects. **RESULTS:** Our search yielded 1,153 articles for treatment patterns and clinical trials on patients diagnosed with Insomnia from PubMed/Medline/Embase/ Cochrane databases. After removing duplicates and non-relevant articles, 65 articles were included for final review. A total of 16 studies had some focus on