

association of Khorasan province (the widest province in Iran). Quality of life was measured with MSQOL-54 instrument. Data was collected by employing a 32-item self-administered questionnaire in a face to face interview. Parametric, nonparametric tests and descriptive statistics analysis were applied ( $p$  value < 0.05). Patients were grouped into three disability stages according to their Expanded Disability Scale Score (EDSS). **RESULTS:** The Patients mean age was 31.78 (SD: 9.67) years, % 73.8 were female and %26.3 were male, and their mean EDSS was 2.4 (SD: 1.26) whereas EDSS increases, the costs also increases, which is a positive correlation. The mean QOL was 0.54 that as QOL increases, the costs decreases, which is a negative correlation. The MS medications (Interferon) have a cost around \$ 46625 per year for each patient that are subsidized about \$ 24452 IR by governmental sector. Up to \$ 17104 are paid by insurance and \$ 5263 directly by patients. The costs per patient-year were calculated as \$ 11560) - 27970.5591 (EDSS= 1-2.5), \$ 29916.909-30015.645 (EDSS=3-4.5) and \$34678.776- 34793.22 (EDSS= 5- 7.5). **CONCLUSIONS:** We conclude that the costs are relevant in MS, especially when disability increases. The catastrophic cost has a high burden to patients, society and health care system

## PND32

## WHOLE EXOME SEQUENCING AS A DIAGNOSTIC TOOL FOR COMPLEX NEUROLOGICAL DISORDERS

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**OBJECTIVES:** The primary objective of this study is to elucidate the effect of whole exome sequencing (WES) in diagnosing children with a developmental delay due to unexplained conditions presumed to be genetic. A secondary objective is to collect all resources used by these children to gain insight into the total costs over time for the traditional diagnostic pathway and the additional costs to diagnose a patient using WES. **METHODS:** We included twenty children at the Sylvia Toth Centre (STC) in Utrecht, the Netherlands, who have underwent previously extensive clinical diagnostic workups and for whom no diagnosis was found after the last extensive workup. On all twenty children and parents WES will be performed, thereby obtaining a list of exonic candidate mutations for each patient. In parallel all resources used were collected by assessing the clinical records of patients. These resources were linked to unit costs to obtain the total cost per patient. Total cost per patient was then compared to the cost of care using WES, assessed for each individual patient. **RESULTS:** The diagnostic yield from the 13 patients sequenced thus far is 23% indicating a 23% increase in number of diagnoses compared to the current diagnostic pathway. On average these patients have had numerous visits to the hospital, overnight stays and different diagnostic workups to unravel the genetic cause of their neurological disorder. Total cost of the current diagnostic pathway is therefore up to ten fold higher compared to the total cost of only providing WES. **CONCLUSIONS:** Comparing the diagnosis and costs with and without the use of WES gives a clear picture of the clinical and economic feasibility of putting WES into standard diagnostic practice at the STC and similar genetic centers over the world.

## PND33

## FINANCIAL AND CLINICAL IMPLICATIONS OF INTRAMUSCULAR VERSUS SUBCUTANEOUS INTERFERON BETA-1A IN PORTUGAL, BASED ON THE FINDINGS FROM THE COCHRANE COLLABORATION REVIEW OF FIRST-LINE TREATMENTS FOR RELAPSING-REMITTING MULTIPLE SCLEROSIS

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**OBJECTIVES:** To estimate the clinical and financial impact of Interferon beta-1a intramuscular (IM) and subcutaneous (SC) formulations in Portugal, based on the findings from Cochrane review regarding first-line treatments for relapsing-remitting multiple sclerosis. **METHODS:** An Excel-based model estimated the number of relapses and costs incurred by a hypothetical cohort of 1000 patients treated with two types of interferon beta-1a. The model evaluated the consequences of treatment with SC versus IM interferon beta-1a, as this was the only comparison whose data quality was assessed as 'high' by the Cochrane Review (Filippini 2013). Risk of relapse was based on the 2-year data from the Cochrane meta-analysis. The analysis was performed from a Portuguese National Health Service (NHS) perspective including only direct costs. Although efficacy was kept constant as Cochrane did not report outcomes based on Expanded Disability Status Scale (EDSS), costs of relapse were available for patients with different EDSS values, thus allowing estimation of cost impact for different types of population. **RESULTS:** According to the model, treatment with IM interferon beta-1a is expected to result in a total of 743 episodes of relapse, whereas SC interferon beta-1a is expected to result in a total of 570 cases, less 173 cases, over a 2-year period. Use of the SC formulation in a population with EDSS  $\leq 3$  will result in savings of €690,213.04 over the 2-year period due to avoided relapses. In a more severe population, with EDSS between 3.5 and 4.5, these savings are expected to be €889,865.74 over the same 2-year period. **CONCLUSIONS:** When compared with the IM formulation, the use of SC interferon beta-1a seems to be associated with fewer cases of relapse, resulting over a 2-year period in considerable potential savings to the NHS in terms of relapses avoided.

## PND34

## CLINICAL OUTCOMES AND HEALTH CARE RESOURCE UTILIZATION IN A 1-YEAR OBSERVATIONAL STUDY OF PATIENTS WITH NON-FOCAL DISABLING SPASTICITY WHO ARE RESISTANT OR INTOLERANT TO ORAL THERAPY TREATED WITH INTRATHECAL BACLOFEN THERAPY AT THE INSTITUT GUTTMANN (SPAIN). EPICE STUDY

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**OBJECTIVES:** To assess clinical outcomes, health care resource utilization and associated costs of intrathecal baclofen therapy (ITB) for the treatment of non-focal disabling spasticity (N-FDS) in patients who are resistant or intolerant to oral ther-

apy. **METHODS:** Observational, non-interventional, prospective, single-center study of 1 year follow-up from ITB implant onward. **RESULTS:** 20 consecutive patients with ITB indication were recruited; 13 received an ITB implant during the study period; 1 implant was rejected and thus explanted. 12 patients, of whom 10 had spasticity due to spinal-cord injury, 1 to multiple sclerosis and 1 to adrenoleukodystrophy, provided data for the study and 9 completed follow-up. After 12 months of ITB, mean changes from baseline were: -2.6 on the Penn scale ( $p=0.011$ ), -1.1 ( $p=0.059$ ) and -2.8 ( $p=0.011$ ) on the Ashworth upper and lower scale, respectively and +4.4 on the FIM scale ( $p=0.075$ ). Mean utility gain, assessed with the HUI3 scale, was 0.054 ( $p=0.091$ ) after 1 year. Mean total ITB test and permanent implant cost per patient were €528 and €14,225, respectively. Mean quarterly spending on oral antispastics decreased by €42 at month 12, while consumption of intrathecal baclofen stabilized after 6 months at €39. At baseline, 4 patients received botulinum injections, while none did at the end of follow-up. Catheter-related adverse events occurred in 2 out of 12 patients, with a total mean cost per event of €2,387. While waiting to receive ITB, spasticity-related hospitalizations due to urological complications occurred in 2 out of 20 patients, with a mean cost of € 9,044 per event; no such events were observed after ITB implant. **CONCLUSIONS:** Clinical outcomes of patients with N-FDS improved after ITB. Initial therapy costs were considerable, but were partially offset by savings in drugs and spasticity-related events after 1 year follow-up. Results should be interpreted cautiously because of the small number of observations.

## PND35

## NUEDEXTA FOR THE TREATMENT OF PSEUDOBULBAR AFFECT: ESTIMATING THE FINANCIAL IMPACT TO THE SCOTTISH NHS

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**OBJECTIVES:** Pseudobulbar Affect (PBA) is a neurologic disorder of emotional expression, resulting in frequent and involuntary episodes of crying and/or laughing. Common neurological conditions associated with PBA include: Alzheimer's disease, amyotrophic lateral sclerosis, multiple sclerosis, Parkinson's disease, stroke and traumatic brain injury. Nuedexa® (Avanir Pharmaceuticals Inc.) is the only EMA-approved PBA treatment. The financial impact of introducing Nuedexa to a national health care system, including Scotland, has never been formally estimated. **METHODS:** An Excel® based cost-calculator was developed. Prevalence, epidemiology and mortality estimates for causative neurological conditions as well as PBA prevalence in those conditions were sourced from the literature. Unit costs (drugs, hospitalisation etc.) were taken from national databases and standard care treatment mix and resource use were derived from a US claims database. A range of market uptake rates were used with further sensitivity analyses undertaken. **RESULTS:** The estimated cost of standard care in Scotland for PBA is circa £32.4 million annually (circa 22,500 patients). In year 1 following introduction, 67 patients are expected to receive Nuedexa, resulting in a cost increase by £0.1 million to £32.5 million. By year five, 836 patients are estimated to receive Nuedexa, resulting in a projected total annual cost of £34.6 million. Therefore the estimated annual budget impact of Nuedexa ranges from £0.15 million (year 1) to £1.88 million (year 5). The incremental cost per patient is £2,246. The model was sensitive to changes in uptake rates, cost of background therapy and PBA symptom severity. When patients with moderate to severe PBA symptoms receive treatment, the projected cumulative year 5 budget impact estimate is £7.56 million. **CONCLUSIONS:** The estimated financial impact of introducing Nuedexa in Scotland is modest. Even if more patients are identified, the relatively small incremental cost per-patient of Nuedexa is unlikely to have a major impact on the Scottish NHS.

## PND36

## ANALYSIS OF EXPENDITURE IN MULTIPLE SCLEROSIS DISEASE MODIFYING THERAPIES EVOLUTION BETWEEN 2004-2013 IN SPAIN

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**OBJECTIVES:** To analyze factors of recent evolution of Multiple Sclerosis (MS) Disease Modifying Therapies (DMT) budgets in Spain between 2004 and 2013. **METHODS:** 2004-2013 single DMT monthly expenditure was provided by IMS Health. Monthly and annually evolution of number of patients, billing, drug cost per patient and cost per year of treatment were calculated. Two periods: 2004-2013 and 2007 (start marketing second lines DMT) -2013 period were analyzed for each DMT line. (First line: subcutaneous and intramuscular interferon (IFN)  $\beta$ -1a, subcutaneous IFN  $\beta$ -1b and glatiramer acetate injection. Second line: natalizumab and fingolimod). **RESULTS:** During 2004-2013 DMT expenditure increased from €115.5M to €319.3M due to: A greater number of patients 147% (10.60 % annual growth per year) and a further growth of annual cost per patient: 12% (1.29 % annual growth per year). In December 2013 second lines correspond to a 29.61% of DMT expenditure. Annual cost per patient in second line represents 70% over cost per treated patient and 83% greater than first line DMT cost per year. If year 2007 is omitted from analysis (Only 68 second-line treatments and M1.44€ of associated expense) and is analyzed 2008-2013 period, second-line DMT represent 43% of new treatments causing a 60% increase in DMT expenditure. In 2013 second line DMT participation reaches 64% of new regimens causing the 79% of increase DMT expenditure. **CONCLUSIONS:** The growing incorporation of new therapies and the noticeable rise in the number of treated patients (10.60 % annual growth per year) are components to consider in the pharmaceutical budget management.

## PND37

## HEALTH CARE RESOURCE USE AND COST OF MULTIPLE SCLEROSIS IN SLOVAKIA: RESULTS FROM THE NATIONAL CROSS-SECTIONAL STUDY

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**OBJECTIVES:** Comprehensive economic costs of multiple sclerosis (MS) according to EDSS states can only be assessed by evaluating MS management in real clinical practice. The objective of this cross-sectional study was to measure the