

utilization and costs in individuals with probable DMD. **METHODS:** We identified males aged 5-25 with at least one claim for hereditary progressive muscular dystrophy between 11/01/2011 and 10/31/2013 (date of first claim = index) from a US administrative claims database. Patients were required to be continuously enrolled with pharmacy benefits for 12 months after index date. Cardiac drugs, gastrointestinal drugs, and adrenals (including steroids) were evaluated in the 12 months after index. Number of inpatient and ER admissions, number of non-ER outpatient claims and prescriptions, as well as healthcare costs (overall and DMD-specific) were evaluated in the 12 months after index. **RESULTS:** A total of 1,773 males with probable DMD were identified (mean age 14.5). We observed 368 (31%) patients on adrenals, 356 (30%) on cardiac drugs, and 124 (10%) with gastrointestinal drug use during follow-up. In the year after index, 16% had at least one inpatient admission (13% DMD-specific admissions), and 29% had at least one ER admission (13% DMD-specific). The average number of outpatient claims was 79.8 (33.3 DMD-specific), and average number of prescriptions was 17.8. Mean annual costs were \$34,381 (\$17,581 DMD-specific). **CONCLUSIONS:** Healthcare utilization and costs were substantial, and are likely to be underestimated due to inclusion of milder non-DMD forms of muscular dystrophy in the present study. Although DMD is a rare disease, the health burden and societal costs are considerable. New therapies to address the underlying disease are urgently needed.

#### PND93

##### HEALTH CARE COSTS ASSOCIATED WITH GERIATRIC PATIENTS DIAGNOSED WITH MULTIPLE SCLEROSIS TAKING DISEASE MODIFYING AGENTS IN THE UNITED STATES

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**OBJECTIVES:** Understanding the health care costs associated with Multiple sclerosis (MS) in the geriatric population is not well studied. The objective of this study is to assess the health care costs associated with geriatric patients diagnosed with MS and taking disease modifying therapies (DMTs) in the US. **METHODS:** A large US administrative retrospective claims database was used to identify patients diagnosed with MS and were prescribed DMTs between January 2010 to December 2012 were included in the study. All patients were  $\geq 65$  years of age and continuously enrolled in the same health plan for at least a year. Descriptive statistics and chi-square tests were performed on the data and statistical significance level was set a priori at 0.05. **RESULTS:** There were a total of 88,921 patients that met the study inclusion criteria. Majority (66.9%) of the patients was taking subcutaneous injections (SC), 31.2% were taking IV/IM (IVM) and 1.9% was taking oral (OR) DMTs ( $p < 0.001$ ). Patients on average were charged \$4227.9  $\pm$  2354.2 with a significant difference ( $p < 0.001$ ) between the three drug groups (OR \$5087 vs SC \$4225 vs IVM \$4188). However, the mean allowed amount by the health plan was \$3692.5  $\pm$  1915.5 and the actual paid amount was \$3587.1  $\pm$  1921.5 with a significant difference between the groups ( $p < 0.001$ ). On average, patient's deductible was \$17.9  $\pm$  207.8 and patient co-payment was \$83.0  $\pm$  304.3 with a difference between the groups ( $p < 0.001$ ). For patients whose prescription was on their health plans formulary were charged lower (\$4187 vs \$4246,  $p < 0.05$ ) and paid lower co-payment (\$78 vs \$97,  $p < 0.001$ ) than who were not. There was a significant variation in the cost of the treatments in different regions in the USA ( $p < 0.001$ ). **CONCLUSIONS:** The overall costs for oral DMTs were higher than SC and IVM DMTs.

#### PND95

##### DEVELOPMENT OF MS BUDGET MANAGER: A PRACTICAL TOOL TO ASSIST ALTERNATIVE STAKEHOLDERS AT MULTIPLE DECISION LEVELS IN THE FORECAST AND FINANCIAL MANAGEMENT FOR MULTIPLE SCLEROSIS (MS)

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**OBJECTIVES:** There are currently several pharmaceutical options for multiple sclerosis (MS), some introduced recently and some more coming in the future. They are characterized by different modes of action, schemes of administration and, ultimately, costs. MS resources management is thus complex and rely on proper interaction between stakeholders – hospital pharmacists, MS department heads, hospital top management, local health representatives, heads of regional pharmaceutical policy. This is particularly true in Italy, a federal and delocalised setting comprising local, provincial, regional, and national decision makers. The aim of this work was to design a tool to explore the financial implications and ultimately align decision makers. Informed choices might save resources and support the adoption of innovative treatments, thus contributing to overall sustainability and equity in patient access. **METHODS:** We developed a dynamic user-friendly tool in Microsoft Excel® with VBA® macros. The flow of the analysis is stepwise and comprises, on top of adherence, variables usually excluded from standard budget impact such as intra- and inter-class switches, potential wash out period, as well as drop-out rates. All variables can be modified or excluded by users since default values can be restored at any time. The tool is open and can accommodate future treatments. **RESULTS:** The tool assesses the financial impact of alternative choices in a comprehensive manner since results can be displayed with the desired level of detail – cumulative or annual, overall or by treatment line and/or cost item. **CONCLUSIONS:** This is an example of how to match the required level of information to specific end-users, encouraging both discussion and decision making in the best interest of patients. This is a prerequisite in multifaceted realities like Italy but it could assist in many other settings where stakeholders need to align to each other in order to forecasts accurately budget allocation.

#### PND96

##### HUMANISTIC AND ECONOMIC BURDEN OF FOCAL DRUG-REFRACTORY EPILEPSY IN EUROPE

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**OBJECTIVES:** The aim of this research is to assess the humanistic and economic burden of focal drug-refractory epilepsy in Europe. **METHODS:** A PubMed literature review was performed to identify publications from January 2004 to December 2014 on prevalence and incidence, impact on quality of life and associated costs of epilepsy. **RESULTS:** In Europe around 6 million people have epilepsy, with 30-45% of patients being drug-refractory and 70% of those having focal drug-refractory epilepsy. The prevalence and incidence rate of epilepsy is 457 and 43.87 per 100,000 persons, respectively. Epilepsy is associated with psychiatric comorbidities, chronic somatic conditions, significantly lower quality of life and 2-11 times higher all-cause mortality than the general population. In 2004 health care expenditures for the treatment of epilepsy accounted for 0.2% of the total European national income and the annual cost per patient varied from €2,000 to €11,500. In 2010, the yearly cost of epilepsy ranged from €13.8 to €20 billion. The cost of epilepsy depends on the severity and frequency of seizures and if patients are drug-refractory (20% to 40% of drug-refractory patients account for 80% of the costs). The main cost drivers of epilepsy treatment are hospitalizations, antiepileptic drug costs and indirect costs (due to high unemployment rate; 46% compared with 19% for the matched control population). Standard therapy for drug-refractory focal epilepsy is open surgery which is highly effective but also highly invasive and requires strict screening criteria. Minimally invasive surgical techniques are an alternative to open surgery and have shown promising clinical benefits with lower neurological impairment and less hospital stays compared with open surgery. **CONCLUSIONS:** This data highlights the high humanistic and economic burden of focal drug-refractory epilepsy in Europe, and the need for new procedures to improve health outcomes and reduce health care resource utilization.

#### PND97

##### IMPACT OF ONGOING NATIONAL HEALTH TECHNOLOGY ASSESSMENT CHALLENGES ON PATIENT ACCESS TO NEW THERAPIES FOR MULTIPLE SCLEROSIS IN GERMANY AND THE UK

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**OBJECTIVES:** In recent years, health technology assessment (HTA) agencies have encountered substantial technical challenges in assessing the relative value of new therapies for multiple sclerosis (MS), potentially delaying patient access to promising drugs. We assessed the impact of contrasting approaches to HTA in Germany and the UK on patient access to new therapies for MS. **METHODS:** Technology appraisals performed by the National Institute for Health and Care Excellence (NICE) and Institute for Quality and Efficiency in Healthcare (IQWiG) since the Pharmaceuticals Market Reorganisation Act (AMNOG) in January 2011 were assessed. Quantitative and qualitative assessment of appraisal decisions in the context of overall assessment processes, evidence requirements and anticipated patient impact were conducted for each technology. **RESULTS:** Three therapies have been assessed by both NICE and IQWiG (fingolimod, dimethyl fumarate and teriflunomide); both bodies have assessed additional therapies independently. All three technologies were recommended by NICE in restricted patient populations, with an agreed price discount. NICE faced considerable technical methodological challenges during the assessment process, including comparator selection (blended versus pair-wise incremental analyses), assessment versus non-cost-effective beta-interferon comparators and appropriate use of mixed treatment comparisons. In some cases, modelling assumptions accepted in previous appraisals were criticised by NICE in subsequent submissions. By contrast, IQWiG and the Federal Joint Committee (G-BA) concluded that all drugs provided no proven clinical benefit, except fingolimod (minor benefit in limited subset of patients with rapidly-evolving severe relapsing-remitting MS), due to limited clinical data versus IQWiG-specified comparators. **CONCLUSIONS:** Patients in Germany can access all three therapies, consistent with their marketing authorisations, despite the negative IQWiG appraisal recommendations. In the UK, however, fewer patient subgroups can access the same therapies, partly due to evolving methodologies used by NICE to assess cost-effectiveness. Greater understanding of challenges that impact HTA decisions may facilitate earlier patient access to promising MS therapies.

#### PND98

##### A LONG TERM ANALYSIS OF THE CLINICAL AND COST EFFECTIVENESS OF GLATIRAMER ACETATE FROM THE UK MULTIPLE SCLEROSIS RISK SHARING SCHEME

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**OBJECTIVES:** The primary objective of this analysis was to model the clinical and cost effectiveness of glatiramer acetate (GA) using 6-year data from the UK Multiple Sclerosis Risk Sharing Scheme (RSS). **METHODS:** A continuous Markov model was developed to assess mean Expanded Disability Status Scale (EDSS) and utility at year-6 and to determine whether this was consistent with a cost-effective target of £36,000 per quality-adjusted life year (QALY) projected over 20 years. In populating the model, data from patients fulfilling the Association of British Neurologists (ABN) 2001 guidelines were analysed together with matched patient data from the British Columbia Multiple Sclerosis database (BCMS) as a comparator. Primary outcome was the progression ratio, measured both as EDSS score and utility, determined for the GA treated group by adjusting within the model the theoretical ratio imposed on the comparator patients until the difference between the theoretical and actual treated groups was mathematically zero. This effective ratio was input into the cost-effectiveness calculator part of the model to determine the cost per QALY. **RESULTS:** 978 patients starting GA were enrolled (F: 755, M: 223), with an average age of 30.0 years at entry. 898 BCMS patients were included as comparators. GA patients had a mean follow-up of 5.23 years (SD 1.33). The Markov model showed 30.3% slower EDSS progression for the GA cohort than predicted for untreated controls (progression ratio: 69.7%). Utility ratios were consistent with cost-effectiveness (ratio: 44.2%) for GA. Cost per QALY for GA was well below the target of £36,000. **CONCLUSIONS:**