

year 1, increasing to only 9% in year 5. A higher treatment cost associated with the introduction of pasireotide LAR is partially offset by lower indirect and adverse event costs. Through sensitivity analyses, alternative model inputs showed the budget impact results to be robust. **CONCLUSIONS:** With a low prevalence for this rare disease, the inclusion of pasireotide LAR in the Finnish national formulary for the medical treatment of CD will have a minimal impact on the total budget.

## PSY36

#### ECONOMIC EVALUATION OF RECOMBINANT FACTOR VIII FC FUSION PROTEIN (RFVIII FC) FOR IMMUNE TOLERANCE INDUCTION (ITI) IN PATIENTS WITH HEMOPHILIA A WHO DEVELOP INHIBITORS UNDER THE BRAZILIAN UNIFIED HEALTH SYSTEM (SUS) PERSPECTIVE

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**OBJECTIVES:** Hemophilia A patients with inhibitors may go through long and costly ITI with conventional FVIII. Case report and chart review data have shown the potential of rFVIII FC (ELOCATE®) for rapid tolerization in ITI and therefore may offer cost savings. Our objective is to develop a cost-minimization and budget-impact analysis of rFVIII FC for ITI in hemophilia A patients with inhibitors, under the perspective of Brazilian SUS. **METHODS:** A decision tree was built to replicate the flow of patients treated with ITI, who reach complete response, partial response, or failure. The time horizon considered was 5 years to account for the duration of ITI up to 33 months per Brazilian guidelines. This included additional months of prophylaxis if ITI succeeded. Results were segmented for first-attempt and rescue ITI patients. Probabilities of response, treatment duration, and health resource use for conventional FVIII ITI were obtained from literature (e.g., Valentino et al 2015) and expert opinion. Inputs on rFVIII FC ITI were obtained from real-world data (Ragni et al 2016, Carcao et al 2017). Only direct medical, factor VIII, and bypassing agent costs were considered. Unit costs were obtained from public data of Brazilian federal purchases. Univariate sensitivity analyses were conducted. **RESULTS:** For an average patient, for first-attempt ITI, total 5-year cost with rFVIII FC or conventional FVIII were: R\$1,128,866 and R\$1,141,728, resulting in a cost reduction of R\$12,862 with rFVIII FC; for rescue ITI, total 5-year costs were R\$7,469,753 and R\$8,235,908, resulting in a cost reduction of R\$766,155 with rFVIII FC. For 279 patients (prevalence) and 33 new patients per year (incidence) in Brazil, total 5-year budget impact to Brazilian SUS was savings of R\$13,528,440. The sensitivity analysis showed consistent cost-saving results. **CONCLUSIONS:** The incorporation of rFVIII FC to Brazilian SUS for ITI treatment of hemophilia A patients with inhibitors could potentially lead to significant cost-savings.

## PSY37

#### REIMBURSEMENT OF VEDOLIZUMAB ACCORDING TO THE INDICATIONS: WHAT ARE THE IMPACTS IN THE 37 PUBLIC HOSPITALS OF PARIS?

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**OBJECTIVES:** In France, the reimbursement of hospital drugs according to the indications was implemented: in addition to diagnosis-related group (DRG) tariffs, included into DRG tariffs or no refund. Until January 2017, vedolizumab was refunded from 3rd line in the ulcerative colitis (UC) and in Crohn's disease (CD) in addition to DRG tariffs and was not refunded for 2nd line. Since January 2017, the CD was included into DRG tariffs (€40 estimated drugs cost) and the UC was still refunded in addition to DRG tariffs (€1529). An exceptional financing of €3 million distributed among the French hospitals was implemented for patients initiated in CD before January 2017. This study assesses the impacts of this change on both the prescriptions alterations and the reimbursements for the vedolizumab into 37 Public Hospitals of Paris (APHP). **METHODS:** The data collected were: i) consumptions/expenditures in vedolizumab from 04/2016 to 09/2017; ii) reimbursements from 01/2017 to 09/2017 with the PMSI (French Hospital National Database) and ICD10 diagnosis. **RESULTS:** From 01/2017 to 09/2017, 15/37 hospitals have used vedolizumab with four main hospitals (61% of consumption). 1936 vials (€3,594,677) were prescribed (+22% compared with the 9 previous months) including 47% (912 vials; €1,712,504) for the CD (+18%). A downturn of -29% in the CD was observed between 01/2017 from 09/2017. 1901 vials (€3,419,318€) were reimbursed including €1,693,360 for the CD (46%). The prescription for the CD in the hospitals of APHP has represented 56% of the national exceptional financing of €3 million. **CONCLUSIONS:** The CD still accounts for a large share of prescriptions. The decrease has begun with acceleration since June 2017 with the switches from vedolizumab to ustekinumab (alternative reimbursed in addition to DRG tariffs). Nevertheless, will the hospitals be able to continue to pay for the CD patients in failure of ustekinumab but responders to vedolizumab?

## PSY38

#### ECONOMIC BURDEN OF INFANT-ONSET (TYPE 1) SPINAL MUSCULAR ATROPHY: A RETROSPECTIVE CLAIMS DATABASE ANALYSIS

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**OBJECTIVES:** Spinal muscular atrophy (SMA) is a rare genetic and life-threatening neuromuscular disease. Data on the cost burden of SMA type 1 for US health plans is limited. **METHODS:** A retrospective analysis was conducted to estimate the economic burden of SMA type 1 using QuintilesIMS's PharMetrics Plus Health Plan Claims Database. Infants with ICD-9 codes for SMA ≤1 year old were classified as SMA type 1 (N=119) and matched (1:1) according to age, gender, index year, and Charlson Comorbidity Index with a random sample of infants without SMA type 1.

All-cause healthcare resource utilization (HCRU) and costs (pharmacy, outpatient, and/or inpatient/hospitalization) incurred between February 2011 and November 2016 during the post-index/follow-up period (≥30 days up to 360 days) were compared. Death was assumed if select events (eg, cardiac arrest/failure) occurred during the last month in which claims were available. **RESULTS:** Significantly more SMA infants (98.32%) received ≥1 all-cause pharmacy, outpatient, or in-patient services during the post-index period compared to matched infants (54.62%; P<0.0001). Mean per-patient-per-month (PPPM) all-cause HCRU was significantly higher for SMA infants: pharmacy (1.43 vs 0.37 prescriptions), outpatient (14.10 vs 2.17 services), in-patient (0.23 vs 0.003 admissions) (all, P<0.0001). Mean PPPM hospitalizations (0.23 vs 0.003), length of hospital stay (6.93 vs 0.09 days), procedures per admission (1.49 vs 0.03), and readmissions (0.04 vs 0.00) were also significantly greater for SMA infants (all, P<0.0001). Subsequently, all-cause pharmacy, outpatient, and inpatient costs PPPM were significantly greater in SMA infants (\$371 vs \$20; \$4,192 vs \$232; and \$22,500 vs \$22, respectively [all, P<0.0001]) resulting in extrapolated all-cause annual cost per patient of \$324,751 (SMA) vs \$3,294 (matched). **CONCLUSIONS:** The economic burden of SMA type 1 to US health plans is substantial; a treatment that alters the early natural course of disease is needed and might result in long-term cost savings.

## PSY39

#### ASSESSING HOSPITAL UTILIZATION OF PATIENTS DIAGNOSED WITH MARFAN SYNDROME

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**OBJECTIVES:** Marfan syndrome (MFS) is a rare disorder affecting connective tissue and impacting multiple organ systems. Limited data exists describing hospital utilization in MFS patients. The objective of analysis was to describe MFS patient hospital utilization and examine acute care services in the MFS population. **METHODS:** This non-interventional, retrospective study analyzed a cross-section of MFS-diagnosed patients with hospital visits between October 2015 and September 2017 in the de-identified Vizient health system database, which consists of over 400 hospitals across the United States. **RESULTS:** The study population included 9,279 visits across 4,748 unique patients. Males represented 52.0% of the population. Average age was 35.5 with 22.2% of the population under the age of 18. Nearly 70% of visits were in 300+ bed, teaching facilities and 96% of visits were in urban hospitals Payer type was 39% commercial, 17% Medicaid, 20% Medicare, and 24% other/unknown. The average Charlson Comorbidity Index was 1.2. The most prevalent comorbidities included peripheral vascular disease (PVD, 30.0%), hypertension (22.3%), chronic pulmonary disease (CPD, 18.8%), and congestive heart failure (CHF, 11.7%). Although nicotine dependence was high (13.5%), alcohol dependence was low (1.1%). The most common disease-related complications included aortic aneurysm (11.6%), eye disorders (6.6%), mitral valve disorders (6.6%), scoliosis/kyphosis (6.2%), asthma (6.1%), and aortic ectasia (5.9%). Utilization was primarily outpatient (83.6%) with 31.3% of all visits classified as urgent or emergency. Inpatient length of stay was 7.7 days with an average cost of \$22,472 per visit. Average cost of outpatient visits was \$1,412. Average inpatient payment was \$24,580 while average outpatient payment was \$1,471. In-hospital mortality rate was low with 0.5% overall mortality and 2.4% mortality for inpatiently. **CONCLUSIONS:** MFS patients incur substantial health care utilization and costs.

## PSY40

#### HEALTHCARE COST OF POTENTIAL GLUCOCORTICOID-ASSOCIATED ADVERSE EVENTS IN PATIENTS WITH GIANT CELL ARTERITIS

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**OBJECTIVES:** To quantify the healthcare expenditures associated with oral glucocorticoid-related adverse events (OGCs-AEs), among patients in the US with giant cell arteritis (GCA) using claims data from MarketScan® Commercial and Medicare Supplemental Databases. **METHODS:** Patients age ≥50 years with GCA and at least one OGC prescription fill, during 1/1/2009-6/30/2014 (first OGC claim after GCA diagnosis date = index date) were selected. Cumulative dose of OGCs was measured during the 1-year post-index period. Patients were stratified in four cohorts (>0-≤2,607 mg, >2,607-≤4,800 mg, >4,800-≤7,200 mg, >7,200 mg) based on the distribution of OGC exposure. Incidence of potential AEs and AE-related direct healthcare costs (2016 USD) were also assessed during the 1-year post-index period. A generalized linear model with log link and gamma distribution was used to evaluate the association between the log of cumulative dose of OGCs and AE-related direct healthcare costs, adjusting for baseline characteristics. **RESULTS:** The 1,602 GCA patients (mean age, 73, 69% females) had a mean cumulative OGC dose post-index of 5,806 mg (median=4,800 mg), with most exposure occurring in the first 6 months. The proportion of patients with any potential OGCs-AEs was 36.5% overall (n=584) and increased as cumulative dose increased (30.7%-45.3% across quartiles). Unadjusted mean AE costs for patients with an AE was \$12,818 (median=\$1,844). In the multivariable model, increasing OGC dose was associated with increasing AE-related healthcare costs (cost ratio=1.38 (95% CI 1.16-1.64) per 1 unit increase in log(cumulative OGC dose), p<0.001). Mean (median) predicted AE costs for the dosing quartiles were: \$4,389 (\$2,749) for >0-≤2,607 mg, \$5,176 (\$3,009) for >2,607-≤4,800 mg, \$5,576 (\$3,633) for >4,800-≤7,200 mg, \$6,609 (\$4,447) for >7,200 mg. **CONCLUSIONS:** Rates of OGCs-AEs tended to increase with an increase in cumulative OGC dose, which resulted in increased healthcare costs. These results highlight the need for efficacious therapies that reduce the exposure and potential risks with OGCs.

## PSY41

#### THE ECONOMIC BURDEN OF SPHINGOLOPIDOSIS IN KOREA