Impact of VWF concentrate almost devoid of FVIII as an alternative to FVIII/VWF for
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Methods: A retrospective review of medical charts was conducted via an online physician
survey in France, Germany, Italy, Spain and Sweden).

Results: The low prevalence of patients requiring VWF concentrate ultrasound during the 24 months study period varied from 20.0% in Sweden to 68.0% in Germany. In all countries, except Germany (66.0%), at least 90% of patients had one of the following diagnoses: 1. Painful bleeding episodes 2. VWF deficiency 3. VWD 4. Hemophilia with inhibitors 5. VWF deficiency with inhibitors. Germany had the largest proportion of patients with hospitalisations related to ADPKD (25.7%) whereas Sweden had the lowest (13.3%). Hospital length of stay was longest in Spain (mean, 8.5 days (SD 6.2)) and shortest in the UK (mean, 4.7 days (SD 3.8)). In terms of medication use, 32.7% of German patients were prescribed loop diuretics whereas this proportion was 9.0% in the UK. The UK had the highest rate of opioid use for renal pain overall (8.8%) and among patients on dialysis (27.3%).

Conclusions: This is the first study to provide comparative evidence on MRU in European patients with ADPKD. Results highlight some country-specific differences in treatment patterns, including rates of hospitalisation, frequency of specialist visits and medication use.

PSY50
AN EVALUATION OF MEDICAL RESOURCE UTILISATION IN PATIENTS WITH AUTOSOMAL DOMINANT POLycystic kidney disease in EUROPE
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OBJECTIVES: Autosomal dominant polycystic kidney disease (ADPKD) is the most common genetic kidney disease. Currently there is little published information on medical resource utilisation (MRU) in European ADPKD patients. This study aimed to extrapolate published data to a larger number of patients in Europe and explore differences between countries.

Methods: A retrospective review of medical charts was conducted via an online physician survey in France, Germany, Italy, Spain and Sweden.

Results: The low prevalence of patients requiring VWF concentrate ultrasound during the 24 months study period varied from 20.0% in Sweden to 68.0% in Germany. In all countries, except Germany (66.0%), at least 90% of patients had one of the following diagnoses: 1. Painful bleeding episodes 2. VWF deficiency 3. VWD 4. Hemophilia with inhibitors 5. VWF deficiency with inhibitors. Germany had the largest proportion of patients with hospitalisations related to ADPKD (25.7%) whereas Sweden had the lowest (13.3%). Hospital length of stay was longest in Spain (mean, 8.5 days (SD 6.2)) and shortest in the UK (mean, 4.7 days (SD 3.8)). In terms of medication use, 32.7% of German patients were prescribed loop diuretics whereas this proportion was 9.0% in the UK. The UK had the highest rate of opioid use for renal pain overall (8.8%) and among patients on dialysis (27.3%).

Conclusions: This is the first study to provide comparative evidence on MRU in European patients with ADPKD. Results highlight some country-specific differences in treatment patterns, including rates of hospitalisation, frequency of specialist visits and medication use.

PSY51
COST-CONSEQUENCES ANALYSIS OF THE LONG-TERM PROPHYLAXIS IN A TYPE 1 VON WILLEBRAND PATIENT WITH RECURRENT BLEEDINGS IN ITALY
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OBJECTIVES: Von Willebrand Disease (VWD) is the most common inherited bleeding disorder, caused by a deficiency or abnormality of the Von Willebrand factor (VWF). Long-term prophylaxis with FVIII concentrates is used to prevent or minimize bleeding events. Rituximab is used for patients with severe VWD however, FVIII/VWF is not enough effective in some cases. Furthermore repeated infusions of FVIII/VWF may increase the risk of thromboembolic events. The objective of the analysis was to assess the economic impact of VWF concentrate almost devoid of FVIII as an alternative to FVIII/VWF for

a patient on LTP with recurrent bleedings. METHODS: A cost-consequences analysis was adapted to assess the economic impact from the NHS and Social perspectives. The cost analysis was based on one patient case (type 1 VWD, recurrent gum bleedings), treated with FVIII/VWF and then with FVIII concentrate almost devoid of FVIII. The costs included direct costs (drug acquisition, hospital admissions, outpatient visits and blood tests) and indirect costs (working days lost and wellbeing costs).

RESULTS: The analysis showed a reduction of the number of bleedings when treating with FVIII concentrate almost devoid of FVIII (30 vs 0), minimizing the cost per bleeding episodes (825,965 vs 0), hospitalizations (3,252 vs 0), and remaining 10.7% of working days lost (26 vs 2). The annual health care costs and indirect costs avoided were €94,689 and €3,734 respectively.

Conclusions: The replacement therapy with FVIII concentrate almost devoid of FVIII is one of the possible solutions identified for the hospital resource and number of working days lost per bleedings together with the discomfort related to bleedings.

PSY52
COST PER RESPONSE ANALYSIS FOR THROMBOPOIETIN RECEPTOR AGONISTS (TPO-RA), IN THE TREATMENT OF ADULT CHRONIC IMMUNE THROMBOCYTOPENIA (ITP) IN MEXICO
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OBJECTIVES: TPO-RAs, represent an evidence-based treatment option in the public health system for adult patients with chronic ITP in Mexico. TPO-RAs have not been directly compared in head-to-head randomized controlled trials (RCTs); however, an indirect comparison was undertaken using Bayesian metaregression: the overall plasmapheresis treatment arm was higher in patients receiving Romiplostim than in those receiving Eltrombopag, estimating an Odds ratio (OR) of eltrombopag vs romiplostim as 0.15 (95% CI 0.02, 0.84). The objective of this study is to compare the mean health economic response of TPO-RAs in Mexico, by means of cost per response analysis was developed. Dose was derived from RCTs and Summary of Product Characteristics. Median dose for romiplostim was 2.5 mcg/kg/week and average dose for eltrombopag was 55 mg/day (21.5% of patients received 52.5mg, 41.75mg, and remaining 37.5%-50mg). Costs for romiplostim were based on costs used, considering a representative Mexican patient (65kg), for eltrombopag, it was based on milligrams needed. Cost assessment included cost of medication, cost of administration and in case of eltrombopag, cost of liver monitoring, expressed in Mexican pesos. Crude Overall Response Rate (ORR) for romiplostim was 83%. Placebo adjusted OR for eltrombopag was 42% calculated by applying the OR estimated from the Bayesian indirect comparison performed by the NICE Evidence Review Group. RESULTS: Romiplostim generates a cost per overall platelet response of $219,690.80, while eltrombopag yields $374,137.72. CONCLUSIONS: Within the TPO-RAs, romiplostim generates a lower cost per response than eltrombopag, in adult patients with chronic ITP in Mexico.

PSY53
COST-EFFECTIVENESS ANALYSIS OF BELIMUMAB IN THE TREATMENT OF ADULT SYSTEMIC LUPUS ERYTHEMATOSUS (SLE) PATIENTS WITH POSITIVE BIOMARKERS IN SPAIN
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OBJECTIVES: Belimumab is a novel biological treatment specifically developed for the treatment of active, autoantibody positive SLE patients. The purpose of this study is to assess the cost-effectiveness of belimumab treatment of SLE patients from the Spanish societal perspective. METHODS: A UK cost-effectiveness microsimulation model was adapted to the Spanish setting. The analysis compared standard of care (SoC) vs. belimumab plus SoC with 2 years maximum treatment duration and a life time horizon. Disease activity reduction seen with belimumab in the BILLY-52 and BILLY-76 trials, was extrapolated using data from the US Johns Hopkins Lupus Cohort to predict impact on long-term organ damage and mortality. Utility values and unit direct costs (treatment, administration, patient’s follow-up and organ damage costs) were obtained from UK and Spain published data, respectively. Indirect costs were calculated using the Human Capital Approach method. A discount rate of 3% was applied to costs and outcomes. Results were expressed as 1QALY. Deterministic and probabilistic sensitivity analyses (PSA) were conducted to determine the robustness of the results. An additional analysis was conducted to compare societal and Spanish National Health System (NH) perspectives. RESULTS: The cost per life year gained (ICER) and cost per QALY (ICUR) for Belimumab were €16,647 and €23,158 respectively. In 68% of scenarios plotted in the PSA, belimumab was a cost-effective alternative considering a €30,000/QALY threshold. From NHS perspective (indirect costs excluded), ICER and ICUR were €25,619 and €35,660. CONCLUSIONS: Base-case results show that belimumab is cost-effective from the Spanish societal perspective. From the NHS perspective, the model provides results that fall within an acceptable threshold considering the prevalence and the severity of the disease. These results highlight the importance of adopting a societal perspective, especially in pathologies such as SLE which affect young people of working age.

PSY54
COST-EFFECTIVENESS ANALYSIS OF MAINTENANCE TREATMENT WITH RITUXIMAB IN PATIENTS WITH FOLLICULAR LYMPHOMA RESPONDING TO FIRST LINE INDUCTION THERAPY IN PORTUGAL
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OBJECTIVES: Evaluate the efficiency of rituximab in maintenance treatment of patients with follicular lymphoma (FL) who responded to the first line induction with rituximab versus observation in Portugal. METHODS: Cost-effectiveness (Life Cycle Analysis) with Y 50-80, Y 0-49, Y 0-49 +15, costs and effects extrapolated to 10 years. Outcomes: OS, PFS and EFS survival.