decreased while PGA increased. A 1998–2003 linear extrapolated market share would have predicted a crossing of the two curves around 2005, reinforced by the new 1st line indication of PGA authorized by the EMEA in 2002. This did not occur which might be explained by the non-reimbursement of PGA for first line therapy in Italy. Accordingly, yearly Padova area drug spending was €150,000 less than predicted, on a €600,000 yearly budget. The comparison of IMS data shows two countries where the BB are still prescribed more than PGA, Italy and Germany. The latter is a country where physicians’ fees are reduced if their glaucoma prescription costs exceed their government set budget. PGA monotherapy treatment persistence was longer than with BB, according to Padova and UK GPRD data, in PGA-naive patients. This holds true for first line and second line treatment (UK GPRD); the persistency of a second line PGA equaled first line BB treatment. A short treatment persistency is known to be associated with high cost and disease progression. CONCLUSIONS: Health care regulation impacted glaucoma prescribing and might be one of the reasons for differences observed between the Five European countries. Evaluations of both the cost consequences beyond the drug budget and the public health impact should always accompany the establishment of health care regulations.

SENSORY SYSTEMS DISORDERS—Conceptual Papers & Research on Methods

DERMATOLOGY LIFE QUALITY INDEX IS MORE SENSITIVE THAN PSORIASIS AREA AND SEVERITY INDEX TO MEASURE TREATMENT EFFECT IN PATIENTS WITH PSORIASIS: FINDINGS FROM THE PHOENIX I TRIAL

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OBJECTIVES: This analysis evaluated whether the Dermatology Life Quality Index (DLQI) and the Psoriasis Area and Severity Index (PASI) have different responsiveness to measure change in treatment effect in moderate to severe psoriasis patients. METHODS: In PHOENIX I, 766 patients were randomized to ustekinumab 45 mg or 90 mg at weeks 0 and 4 and then q12 weeks thereafter, or placebo at weeks 0 and 4 with crossover to ustekinumab at week 12. Ustekinumab-randomized patients achieving PASI75 response at weeks 28 and 40 were re-randomized at week 40 to continue maintenance ustekinumab or be withdrawn from treatment until loss of response. DLQI and PASI were assessed at weeks 0, 2, 12, 28, 40 and 52. Multiple regression models were used to assess treatment effect on DLQI by adjusting for PASI improvement. RESULTS: Significantly greater proportions of patients receiving ustekinumab achieved PASI75 response (66.7%) and clinically meaningful improvement (≥5 points) in DLQI (67.8%) compared with placebo (3.1% and 6.0%, each p < 0.001) at week 12. There was a significant correlation between the change in DLQI and change in PASI (r = 0.65, p < 0.001). After adjustment for baseline DLQI, baseline PASI, and change in PASI, ustekinumab was still associated with significant improvement in DLQI (p < 0.001). For patients originally randomized to ustekinumab, the median % improvement from baseline was higher in DLQI (37.5%) than in PASI (21.4%) at week 2, with 11.4% of patients achieving a DLQI score of ≥1, but only 1% achieving a PASI75 response. At week 28 and 40, the DLQI and PASI achieved and maintained similar improvements of ≥90% from baseline. For those who achieved and maintained a PASI75 response from week 12 through week 40, but lost response at week 52, the median improvement in DLQI decreased more significantly (100% at week 40 to 46% at week 52, a 54% reduction) than PASI that decreased from 88.9% at week 40 to 57.6% at week 52 (35.0% reduction). CONCLUSIONS: The DLQI may be more responsive to change of disease status due to treatment intervention than PASI.

SYSTEMIC DISORDERS/CONDITIONS—Clinical Outcomes Studies

A SYSTEMATIC REVIEW OF THE EFFICACY OF RECOMBINANT ACTIVATED FACTOR VII (rFVIIa) AND ACTIVATED PROTHROMBIN COMPLEX CONCENTRATE (aPCC) IN THE ON-DEMAND TREATMENT OF MINOR TO MODERATE BLEEDING EPISODES FOR HAEMOPHILIA PATIENTS WITH INHIBITORS

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OBJECTIVES: The primary treatment for minor to moderate bleeding disorders in haemophilia patients with inhibitors is either rFVIIa or aPCC. The efficacy of both products has been evaluated in individual studies; however there has not been an overall review and attempt to establish a valid estimate of the effectiveness of rFVIIa and aPCC. We undertook a systematic review of the literature in an attempt to establish robust estimates of the efficacy, speed of bleed resolution, and adverse event profile of both rFVIIa and aPCC. METHODS: We identified 11 open-label cohort studies, six randomized clinical trials, including two head-to-head clinical trials and a meta-analysis. The definition of efficacy varies between these studies, but is usually a composite measure of definite relief of pain, reduction in the size of the haemorrhage, and cessation of bleeding. The individual making the interpretation of efficacy (i.e., the clinician, the patient/caregiver, or a combination of both) and the time from treatment initiation to the recording of the efficacy endpoint also varies across the studies. RESULTS: Overall, estimates of efficacy based on randomized clinical trials using dosing regimens in line with guidelines are higher for rFVIIa (81%–91%) than for aPCC (64–80%). Conclusions from a meta-analysis suggest that treatment with rFVIIa may be associated with a faster time to joint bleed resolution than aPCC due to higher efficacy levels at 12, 24 and 36 hour time points. The results from a comparative trial support the improved efficacy rates associated with rFVIIa compared to aPCC. CONCLUSIONS: In general, the studies do report higher efficacy and bleed cessation rates for rFVIIa than for aPCC; however, the measurement of effectiveness of the agents is open to interpretation due to variety of methods being used to evaluate effectiveness. Further head-to-head trials should incorporate a standardized measurement for defining efficiency.

CONTRIBUTIONS OF THE FABRY OUTCOME SURVEY (FOS) TO ADVANCING THE MANAGEMENT OF FABRY DISEASE

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OBJECTIVES: To review how the Fabry Outcome Survey (FOS), a physician-driven multinational database supported by Shire