

**OBJECTIVES:** Reimbursement decisions are often unsystematic and lack transparency, especially for orphan drugs. The objective of this study was to demonstrate whether multi-criteria decision analysis (MCDA) can support rational and explicit reimbursement decision process for orphan drugs in the Netherlands. **METHODS:** An Analytic Hierarchy Process (AHP) framework was used in which Health Economics students were asked to weigh criteria used in drug reimbursement decisions through a web-based survey. Criteria were identified by a systematic literature review. Three different orphan drugs (alglucosidase alfa in infantile Pompe disease, canakinumab in cryopyrin-associated periodic syndromes and investigational product in rare disease) were also assessed by the students on their performance on these criteria. Criteria weights and performance scores were aggregated to an overall score for each orphan drug. Rank-ordering on overall scores prioritized the reimbursement of the three drugs. The students were also asked to assess the AHP survey on feasibility. **RESULTS:** Nine criteria were identified and categorized in four domains; disease (burden of illness without treatment, life-threatening nature of the disease), drug (availability of other treatments, effectiveness of the drug, side effects and safety of the drug), financial aspects (annual costs of the drug per patient, budget impact, cost-effectiveness) and quality of evidence. The criterion 'life-threatening nature of the disease' was given the highest importance weight and budget impact the least. Alglucosidase alfa for treatment of infantile Pompe disease ranked highest of the three orphan drugs examined, particularly due to its performance in the disease and drug domains. The AHP survey was perceived as difficult by the respondents, which was confirmed by poor values for consistency ratios. **CONCLUSIONS:** Performing MCDA can enable explicit, transparent and auditable reimbursement decision-making for orphan drugs. However, its feasibility and applicability needs further investigation.

#### PSY115

##### ORPHAN AND RARE DISEASES - THE PAYER PERSPECTIVE

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**OBJECTIVES:** To look at the affordability of orphan medications across Europe and whether payer attitudes to high-price medications are changing in the face of rising health care expenditure and tighter budgets. **METHODS:** We conducted an online semi-quantitative survey of 10 European markets and the USA to understand how payers views and attitudes are changing in response to new treatments coming to market for rare and ultra-rare conditions. The payers selected for the survey hold or have held senior positions within their respective market institutions. The USA was included to provide international context to the European results. **RESULTS:** 82% of payers surveyed believe that the current approach to orphan drug pricing is unsustainable in the future and all respondents predict a tougher approach from payers going forward. 73% of payers do not believe that patent expiry alone will free up the necessary space for innovative orphan and ultra-orphan products. 82% of the payers surveyed believed that less than half of all orphan and ultra-orphan drugs coming to market are supported by an adequate evidence base for reimbursement. Although payers view rare diseases as a relatively high priority to fund, they are still behind therapy areas such as oncology and cardiovascular disease. **CONCLUSIONS:** As the financial performance of European countries begins to diverge, so do attitudes towards the funding of orphan medicines. The increasing number of rare diseases is forcing payers to view orphan drugs in a new light and they are becoming increasingly sceptical about the prices charged in relation to the clinical benefit offered. There is space for innovation; and patent expiry is freeing up funds, but rare diseases are competing with other therapy areas for limited budget. The bottom-line is that as rare disease spending becomes a higher proportion of pharmaceutical budgets, payers will take action to curb this trend.

#### PSY117

##### SOURCES OF INFORMATION AND PHARMACISTS' KNOWLEDGE REGARDING RARE DISEASES AND ORPHAN DRUGS: CROSS-SECTIONAL STUDY IN SERBIA

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**OBJECTIVES:** The lack of information and scientific knowledge of rare diseases (RDs) and orphan drug (ODs) could affect the quality of health care delivered to patients suffering from rare diseases. The aim of this study was evaluation of the level of the general epidemiological knowledge among pharmacists regarding RDs and ODs as well as how that knowledge is influenced by information sources, education level and years of experience. **METHODS:** The research design was based on a descriptive cross-sectional study. A questionnaire previously used in a pilot KAP study in Serbia in 2012 was applied. The respondents were 182 pharmacists from public pharmacies in seven of 29 districts in Serbia. Individual level of knowledge was assessed by total number of correct answers from a maximum of 9, and overall knowledge was an average of the individual level of knowledge. **RESULTS:** In total, 155 pharmacists were included in the full analysis set (response rate was 86.3%). Overall, the mean age was 43.4 years, and 94% were women. The average number of information sources regarding RD was 1.7%, and mostly one source out of five was used (56.1%). Pharmacists who were engaged in post-graduate programmes or completed such programmes tended to use more sources of information (69.2%) than those who were not involved in any such programme (41.9%). The mean value of correct answers about pharmacists' knowledge regarding RD and OD was 4 ± 1.77. Most pharmacists (n = 30, 19.35%) replied correctly to 6 questions. **CONCLUSIONS:** The results indicate that years of experience and age among pharmacists do not have influence to the overall knowledge about RD. The positive impact of education was evidently, and for the better pharmaceutical care of RD patients the training of pharmacists to proper use of professional sources of information should be usefully.

#### PSY118

##### BEHAVIOR THERAPY FOR OBESITY TREATMENT CONSIDERING APPROVED DRUG THERAPY – AN UPDATE

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**OBJECTIVES:** Many obesity-associated diseases require intensive medical treatment and are cause of a large proportion of health-related expenditures in Germany. Treatment of obesity includes nutritional, exercise and behavior therapy, usually in combination. The goal of behavior therapy for obesity is to bring about a long-term alteration in eating and exercise habits of overweight and obese individuals. Depending of the severity of obesity, drug treatment may be indicated. To evaluate the clinical and economic effectiveness of behaviour therapy for obesity considering approved drugs reducing weight, a Health Technology Assessment was carried out in the year 2008. This HTA was updated with publications up to 12/2013, along with new developments in behavior therapies and drugs. **METHODS:** A systematic review was carried out using relevant electronic literature databases Publications chosen according to predefined criteria were evaluated by approved methodological standards of evidence-based medicine and health economics systematically and qualitatively. **RESULTS:** Nine randomized controlled trials showed moderate but statistically significant reduction of weight in the intervention groups compared to control groups between 1.1 kg (at month 4) and 6.6 kg (at month 9). Studies with several examination time points resulted in statistically significant differences in the first evaluation time point (month 6) but not in the subsequent time points (month 12, 18, 24). The most frequent approach used for behavior therapy, was per phone or Email, two studies offered behavior therapy face-to-face. New behavior therapy approaches applied were techniques such as "Motivational Interviewing" and "Transtheoretical model". No study was identified examining behavior therapy in combination with approved drug therapy. Two identified studies evaluating cost-effectiveness of behavior therapy per Email or phone showed cost-effectiveness for this kind of intervention but the results are biased due to a high rate of drop-outs. **CONCLUSIONS:** Behavior therapy considering new approaches is an effective method to reduce weight.

#### PSY119

##### COMPARISON OF TREATMENT PATTERNS AND DISEASE SEVERITY AMONG PATIENTS WITH PSORIATIC ARTHRITIS (PSA) RECEIVING THEIR FIRST BIOLOGIC, TREATED BY RHEUMATOLOGISTS AND DERMATOLOGISTS IN EUROPE (EU)

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**OBJECTIVES:** To compare rheumatologists and dermatologists in terms of treatment patterns and disease severity among PsA patients receiving their first biologic in 5EU (UK/Germany/France/Italy/Spain). **METHODS:** A medical chart-review study of psoriasis and PsA patients was conducted among rheumatologists and dermatologists in hospitals and private practices to collect de-identified data on disease and treatment characteristics. Physicians were screened for duration of practice (3-30yrs) and patient volume ( $\geq 2$  Psoriasis/PsA biologic patients/month) and recruited from a large panel to be geographically representative in each country. Physicians abstracted charts of the next 5 consecutive Psoriasis/PsA patients in their respective sites. Treatment patterns and disease severity among PsA patients on their first line of biologic therapy treated by rheumatologists and dermatologists respectively were compared using descriptive statistics. **RESULTS:** In Q42012, 337 rheumatologists abstracted 527 PsA patient-charts (mean-age: 47.4yrs, male: 51.4%) and 225 dermatologists abstracted charts of 109 psoriasis patients with PsA (mean-age: 49.0yrs, male: 56.0%; 55.1% were managed in conjunction with a rheumatologist; 67% were referred by GP/another dermatologist). Time to first biologic since diagnosis was 41.0mo/20.8mo for the rheumatologist/dermatologist-treated cohorts; disease severity at biologic initiation per physician judgment was (mild/moderate/severe): rheumatologist-treated-cohort: 2.7%/60.2%/37.2%, dermatologist-treated-cohort: 1.96%/46.08%/51.96%. In rheumatologist-treated-cohort: treatment naïve-12.9%, non-biological DMARDS-experienced: 74.0%, dermatologist-treated cohort: treatment naïve-6.5%, 1-or-2 systemic-treatment-experienced prior to their first biologic-initiation: 69.5%, 38.7%/61.3% and 27.5%/72.5% had moderate-severe/remission-mild disease-status among rheumatologist- and dermatologist-treated cohort respectively. Average current PASI score was higher among rheumatologist-treated-cohort (18.7 vs. 10.3). **CONCLUSIONS:** Across the EU5, PsA treatment patterns and disease severity varied based on physician specialty (rheumatologist vs. dermatologist); at biologic initiation dermatologists reported a significantly higher proportion of moderate/severe PsA patients than rheumatologists, but once first biologic treatment was well established dermatologists reported a lower disease burden than rheumatologists. Factors influencing these observed variations, including optimal therapeutic approaches and care coordination between specialties to alleviate patient burden may warrant further scrutiny.

#### PSY120

##### VARIATIONS IN TREATMENT PATTERNS AND DISEASE SEVERITY AMONG PATIENTS WITH PSORIASIS RECEIVING THEIR FIRST BIOLOGIC THERAPY IN EUROPE (EU)

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**OBJECTIVES:** To assess treatment patterns and disease severity of psoriasis patients receiving their first biologic-therapy in EU. **METHODS:** A medical chart-review study of psoriasis patients was conducted in 4Q2013 in EU5 (UK/Germany/France/Italy/Spain) among dermatologists to collect de-identified data on disease/treatment characteristics. Physicians were screened for duration-of-practice (3-30yrs) and patient-volume ( $\geq 2$ psoriasis biologic patients/month) and recruited from a large panel to be geographically representative in each country. Physicians abstracted the charts of next 5 consecutive psoriasis patients in their center/practice. Results from patients on their first biologic treatment were analyzed and comparisons made to EU5-averages. **RESULTS:** 877 patient-charts were abstracted; 702 (80.0%) were on their first biologic (mean age: 47.3yrs, male: 64.1%). Prior to initiating biologic therapy, immunomodulators/phototherapy were more widely used in UK than in other countries (90.3%/43.1% vs. 76.6%/30.4% overall, respectively); in Germany, fumarates/