OBJECTIVES: BTcP is associated with a number of problematic physical, psychological/emotional and social complications which themselves are not only a relevant source of additional morbidity in these patients, but are also responsible for significant economic implications, for the patient, their relatives, for the healthcare system and society. **METHODS:** We performed a systematic literature search of PubMed electronic database through December 2014 and February 2015. The key words included "breakthrough cancer pain" and "cost effectiveness" or "economics" or "pharmacoeconomic," and MeSH terms included "breakthrough pain" and "neoplasms", or "economics" or "cost-benefit analysis" . There were no language or study design restrictions. A total of 20 articles were identified. Among the 10 included studies, there were two quality improvement projects, two survey studies, three decision-analytic models, one literature review, two pharmacoeconomics studies and one health economic framework. **RESULTS:** BTcP is associated with increased medical costs: direct medical costs, indirect costs, intangible cost. Direct medical costs might include hospital charges, cost of analgesics and other medications, radiotherapy, surgery, and clinician time; direct non-medical costs might include the cost of gas to drive to the clinic, parking during a clinic visit, highway tolls, overnight lodging for family/caregivers, meals for caregivers. Intangible costs include the patient's pain, suffering, depression, anxiety, loss of sleep, and fatigue, as well as the family's and/or caregiver's distress. **CONCLUSIONS:** Additional studies are needed to evaluate quality-of-life improvements and the cost-effectiveness of more systematic analgesic treatments for BTcP. By delineating all the factors describing the impact of BTcP on patients, providers, and society, a comprehensive health economic model will improve clinical decision making, optimize outcomes, and enhance satisfaction with the processes of care across all stakeholder levels. In the case of HTA in breakthrough cancer pain, ethical evaluations appear to be the most important assessment focus among the considered ones.

PSY43

COST OF FAMILIAL MEDITERRANEAN FEVER (FMF) DISEASE IN TURKEY

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OBJECTIVES: Familial Mediterranean Fever disease (FMF) is an autosomal recessively inherited disease characterized by recurrent, self-limited febrile attacks with serositis, synovitis, and occasionally skin involvement. AA amyloidosis is the most serious complication of FMF and can be life-threatening. Daily colchicine is considered standard of care, and is expected to prevent attacks and amyloidosis in most patients. The objective of this study is to estimate the cost burden of FMF in Turkey from payer perspective. **METHODS:** Delphi technique was applied to determine the type and the amount of resources used in FMF based on physicians views. The Delphi method solicits the opinion of an expert panel through a carefully designed questionnaire which in this case included questions on: type, frequency and duration of the health care resources used for diagnosis and treatment of the disease, epidemiology of the disease and colchicine resistant patients. Ten key opinion experts were involved in the study.The responses were analysed and discussed in a face to face meeting followed by consensus building steps. Unit costs of resources used were obtained from payer' Reimbursement Guideline-List of Procedure Fees Per Service. **RESULTS:** The prevalence of the disease is estimated 0.1%. 65% of patients are responders to colchicine treatment, 30% are partial responders and 2-5% are colchicine resistant. The cost of diagnosis was 549,59TL, cost of standard treatment was 3.233,86TL, cost of treatment for colchicine resistant patients was 69.254,32TL per patient. The cost of treatment of two major complications, amyloidosis and renal failure were estimated as 8.628,98TL and 16.120TL respectively. The annual total cost of the disease to the SSI was found as 547.945.305TL without complications and 750.921.801TL with complications. **CONCLUSIONS:** FMF is highly prevalent in Turkey and there are unmet needs for colchicine resistant patients. The burden of disease is increasing with long term complications and colchicine resistance.

PSY44

SOCIETAL AND NON-HEALTHCARE COSTS ASSOCIATED WITH ATYPICAL HAEMOLYTIC URAEMIC SYNDROME IN THE UK: RESULTS OF A PATIENT SURVEY

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BACKGROUND: Atypical haemolytic uraemic syndrome (aHUS) is a progressive, life-threatening, and ultra-rare disease with an estimated prevalence of 5.5 persons per million in England (AGNSS in 2012). **OBJECTIVES:** There is a lack of literature describing the economic and humanistic burden of aHUS. A patient survey was undertaken to evaluate the impact of aHUS on patients, their carers and society in the UK. **METHODS:** aHUS patients and their carers were recruited by the UK aHUS Patients and Families Support Group and were asked to complete an online questionnaire. Respondents described the impact of aHUS on daily activities, work and out-of-pocket expenses. **RESULTS:** 37 patients with aHUS completed the survey. 23 patients were above 18 years of age (23/35). 61% of adult patients (14/23) were employed, 39% (9/23) were not currently in employment; of these, 33% (3/9) were retired. The rate of economically inactive adults among aHUS patients exceeded the UK national average by 20%. Based on the UK median earning (ONS 2012), an annual lost productivity cost of £2,650 per aHUS patient or £472 of lost tax revenues was calculated. 54% (20/37) of aHUS patients had a carer, of which 82% (16/20) were informal carers (ie. family members). On average, carers lost 18 hours of paid work per week and estimated £12,190 lost productivity/£2,170 lost tax revenues per carer. The following out-of-pocket expenses (per patient per year) were estimated using the survey results and findings from the literature: cost of travel to receive healthcare (£780), household expenses (£136), cost of moving home (£65) and lost accommodation costs (£214). **CONCLUSIONS:** Patient survey results showed that aHUS has a substantial economic burden to patients, their carers and society. The total economic burden from lost productivity of patients, carers and out-of-pocket expenses was estimated to be £9,243 per aHUS patient per year.

PSY45

THE BUDGET IMPACT OF ORPHAN DRUGS IN THE US AND CANADA: A 2007-2013 MIDAS SALES DATA ANALYSIS

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OBJECTIVES: In the United States (US), the 1983 Orphan Drug Act (ODA) established incentives for the development of drugs that treat rare or orphan diseases (defined as diseases affecting < 200,000 Americans). Health Canada is currently developing an orphan drug framework (for diseases affecting ~5 in 10,000 Canadians). This study measured total annual expenditures of orphan drugs in the US and Canada (2007-13) and estimated future (2014-18) orphan drug expenditures. **METHODS:** Branded, orphan drugs approved by the US Food and Drug Administration (FDA) between 1983-2013 were identified (N=356). The Canadian analysis only included US orphan drugs with the same orphan indication(s) approved in Canada. Adjustment via disease factoring was applied to products with multiple indications (both orphan and non-orphan) using available data sources to isolate orphan-indication sales only. The IMS MIDAS database was used to assess total orphan drug expenditures, calculated annually from 2007-2013 and evaluated as a proportion of total annual pharmaceutical drug expenditures (all reported in 2014 US dollars). **RESULTS:** Between 2007-2013, expenditures were measured for a final N=316 and N=147 orphan drugs in the US and Canada, respectively. In the US, total orphan drug expenditures accounted for $15.0-$30.0 billion in 2007-13, representing 4.8-8.9% of total U.S. pharmaceutical expenditures, respectively. In Canada, orphan drug expenditures totaled $557.7-$1,005.8 million in 2007-13, representing 3.3-5.6% of total Canadian pharmaceutical expenditures, respectively. Future trend analysis (2014-18) suggests a slowing down in growth of orphan drug expenditures. **CONCLUSIONS:** While the number of available orphan drugs and associated expenditures increased over time, these drugs benefit many people with previously underserved rare conditions. From a societal perspective, the overall budget impact of orphan drugs is small and has remained fairly stable relative to total pharmaceutical expenditures. Concerns that growth in orphan drug expenditures may lead to unsustainable drug expenditures do not appear to be justified.

PSY46

COMPARING THE COST-EFFECTIVENESS OF APCC VS RFVIIA IN ON-DEMAND TREATMENT OF BLEEDS IN HEMOPHILIA A PATIENTS WITH INHIBITORS: A BRAZILIAN PUBLIC HEALTH SYSTEM PERSPECTIVE

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OBJECTIVES: The development of inhibitors is the most challenging complication of hemophilia treatment. Bypassing agents (i.e. aPCC and rFVIIa) remain the therapy of choice but account for a significant proportion of treatment cost. This study aims to compare the cost-effectiveness of aPCC vs. rFVIIa in on-demand treatment from the Brazilian public health system perspective. **METHODS:** A literature based cost model was developed based on utilization and efficacy (evaluated at 2, 6, 12, 24, 36 and 48 hours after infusion) data from the FENOC (head-to-head) trial which investigated the equivalency of the clinical efficacy of aPCC and rFVIIa in on-demand treatment. The median (range) treatment dose was 84.6 (51.5-100) U/kg and 212.5 (98.6-261.8) µg/kg for aPCC and rFVIIa, respectively. Hemostatic efficacy ranged from 75% to 97.6% for aPCC and 60.4% to 85.4% for rFVIIa. Bleed resolution rate also ranged from 53.2% to 95.1% for aPCC and 38.3% to 92.7% for rFVIIa. Cost analysis was limited to drug costs and prices were obtained from the 2014 Brazilian Official Diary: U$0.71/U and U$0.64/µg for aPCC and rFVIIa, respectively. One-way sensitivity analyses were performed to determine model robustness when dose and price were varied by ±25%. **RESULTS:** The cost/kg per on-demand bleed treatment was 55.9% lower for aPCC compared to rFVIIa. The cost/kg per treatment efficacy was 64% to 79% lower for aPCC compared to rFVIIa. Similarly, cost/kg per resolved bleed was 68% to 81% lower for aPCC compared to rFVIIa. Results remained robust for all one-way sensitivity analyses conducted. Given, an estimated total inhibitor population of 684 in Brazil, total saving with aPCC can vary from U$13.2-U$53.2 million depending on market share. **CONCLUSIONS:** aPCC was the dominant therapy in on-demand treatment. Using the current Brazilian prices, for each bleed treated with rFVIIa about 2.3 bleeds could be treated with aPCC at comparable cost.

PSY47

ANALYSIS OF DIRECT COSTS ASSOCIATED WITH CYSTIC FIBROSIS IN SINGLE CENTER IN SLOVAKIA: 5 YEARS REVIEW

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OBJECTIVES: Treatment of Cystic Fibrosis (CF) as rare disease is generally perceived as highly expensive, but we did not have more specific national or local costs data. Since patients are on lifelong treatment we tried to analyzed direct costs associated with CF in single center. **METHODS:** We analyze direct costs associated with treatment management of patients with CF in selected center in Slovakia from 2009 till 2013. We reviewed 182 adult patients regularly visiting CF centrum. We distributed patients according to age that is closely connected with CF prognosis. We included: treatment costs, healthcare procedures, medical devices and hospitalization costs. We used perspective of healthcare provider. We chose cost-of-illness, prevalence-based approach and compared costs based on time/year, gender, age and type of procedure. **RESULTS:** We have found that adult patients with CF between age 30 and 35 (21 patients) consumed most resources from 2009 till 2012, but most significantly