OBJECTIVES: Friedreich's ataxia (FRDA) is a rare inherited neurological disorder affecting approximately 2,000 individuals in the UK. The disease is characterised by debilitating comorbidities such as severe and progressive mobility loss, visual and audio impairments, diabetes mellitus and cardiac abnormalities, which often result in premature death. The disease incurs lifelong direct medical costs and has a significant economic impact on FRDA patients and their families. This study aimed to evaluate the cost of illness (COI) from a service provider (National Health Service, NHS) and societal perspective of all patients with FRDA in the UK. **METHODS:** The treatment pathway was developed based on published FRDA guidelines and expert opinion. A pragmatic literature review, NHS Reference Costs (2014–2015) and the British National Formulary informed model inputs, in conjunction with patient questionnaires and clinician interviews. The model considered two perspectives: (1) direct costs to the NHS including treatment costs and consultations; (2) indirect societal costs to FRDA individuals and social services. **RESULTS:** Overall, the total COI of FRDA patients to the NHS was £8,038,645 per year, with a mean annual cost per patient of £3,556. Loss of mobility and cardiac abnormalities caused the most substantial direct costs: contributing 35% and 21% respectively. Inclusion of societal costs contributed an additional £7,633,263 per year and an additional mean annual cost of £3,376 per patient. Travel costs and loss of earnings to carers were considerable contributors to societal costs. **CONCLUSIONS:** A substantial annual per patient cost of FRDA to the NHS was found, with high societal costs demonstrating an additional economic burden to patients, their families and society as a whole. The development and delivery of new treatment options, particularly to address the loss of mobility and cardiac abnormalities experienced by FRDA patients, will substantially reduce the economic burden of this rare and devastating disease.

PSY58

ECONOMIC BURDEN OF PRIMARY IMMUNODEFICIENCY (PIDD) IN TURKEY

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OBJECTIVES: Aim of our study is to assess the economic burden of severe combined immunodeficiency (SCID) and some other PIDD subgroups in Turkey, from the payer perspective (SSI). **METHODS:** The cost of illness (CoI) methodology was used and only direct costs were included. Because of lack of data, indirect and intangible costs haven't been taken into consideration. The annual cost of PIDD treatment per patient was collected from 39 PIDD patient records, from 5 university hospitals. The real world data includes follow up visits, pharmecutical requirements, screening and laboratory tests, hospital stay, interventions, complications and stemcell transplantation since diagnosed. Early diagnosed patient and late diagnosed patient were analysed separately. Total economic burden was analysed based on disease incidence rate. Calculations were made in TRY and converted to $ using the current currency (June 2016). **RESULTS:** The annual average cost of disease was calculated for SCID (N=20) as $31,892.2; Combined Immunodeficiency: $42,767.06; MHC ClassII:$79,735.3; Omenn Syndrome:$31,600.41; Antibody Deficiency:$19,044.34; Ade nosine Deaminase Deficiency:$180.955; Chronic Granulomataus Disease:$16,680.03; Ataxia Telangiectasia:$21,379.82 per patient. Number of live infant birth was 1,337,504 in Turkey (2014 official census). The incidence rate of PIDD and SCID were 30,5/100000 and 1/10000, respectively. PIDD and SCID patients were calculated as 408 and 133, respectively. Based on the average cost per patient, the burden of PID was $16,077,113 per year. The other important results were late and early diagnosed SCID average patient costs: $77,348.96 and $37,181.38 per year respectively where early diagnosis can lead to $40,167.58 cost savings. **CONCLUSIONS:** Although the prevalence of PID isn't well documented, it can be estimated by current disease rate. Therefore, the economic burden of PID may be higher than estimated. Routine newborn screening for PIDD is proposed for Turkey to lower the economic burden of disease by early diagnosis.

PSY59

BURDEN OF CONGENITAL HYPERINSULINISM IN THE UNITED KINGDOM: A COST OF ILLNESS STUDY

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OBJECTIVES: The economic burden of congenital hyperinsulinism (CHI)—a rare genetic disease causing persistent hypoglycaemia, typically in newborns—has not been well-studied or reported. In patients with the diffuse variant of the disease, management often involves near-total surgical removal of the pancreas, leading to type 1 diabetes mellitus (T1DM) in later life. The aim of this study was to estimate the cost of illness (COI) from a service provider perspective (National Health Service, NHS) of all CHI patients in the UK, and to explore the distribution of the COI within CHI. **METHODS:** The model was based on standard practice of two centres of excellence in CHI. Model inputs were informed by a pragmatic literature review in conjunction with NHS Reference Costs and the British National Formulary (2014–2015). Only direct costs to the NHS were considered. A prevalence-based approach was used and annual costs incurred at all ages were calculated. A deterministic sensitivity analysis (DSA) was run at 5% to identify major cost drivers. **RESULTS:** In total, the COI of all patients with CHI to the NHS was £4,561,827.58 per year. The average cost per patient was £1,468.24. The distribution of costs was skewed among CHI patients, with just 3.1% of all CHI patients (95 patients in their first year of life) contributing to 42.1% (£1,919,751.79) of the total costs. Results from the DSA identified lack of response to first-line therapy, and the development of T1DM post-surgery (and the associated healthcare costs) as major cost drivers in the model. **CONCLUSIONS:** Despite being a rare disease, the annual cost of CHI to the NHS was found to be substantial. Development and management of post-surgical T1DM as a major cost driver highlights the need for effective treatments which could potentially mitigate such consequences and costs.

PSY60

DISEASE ACTIVITY, QUALITY OF LIFE AND INDIRECT COSTS ASSOCIATED WITH ULCERATIVE COLITIS IN POLAND

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OBJECTIVES: The aim of this study was to assess the indirect costs, health-related quality of life, and clinical characteristics of patients with ulcerative colitis (UC) measured by an UC disease activity index in Poland. **METHODS:** A questionnaire survey was conducted among patients with UC. The Patient Simple Clinical Colitis Activity Index (P-SCCAI) was used to assess disease activity and the modified Work Productivity and Activity Impairment Questionnaire was used to assess productivity loss. Demographic data (age, sex, work status, disease onset, place of living) were also collected. Quality of life was presented as utility calculated using the EQ-5D-3L questionnaire. Indirect costs were assessed using the human capital approach, and both absenteeism and presenteeism were taken into account. **RESULTS:** The analysis was performed based on the answers of 225 adult patients with UC. The mean patient age and age at disease onset were 33.03 years (SD: 9.89) and 26.37 years (SD: 8.88), respectively. In the whole study group, the mean P-SCCAI score was 8.45 and the mean utility was 0.8612. The corresponding values for the group of working patients were 8.17 and 0.8740. Utility was significantly higher among patients with disease in remission compared with those with active disease (0.94 vs 0.83, $p<0.05$). A similar association was observed among working patients with UC. The mean number of working hours missed due to the disease was 210.96 per year and mean on-the-job productivity loss was 19.57%. The average annual indirect costs of absenteeism and presenteeism per working person were 7,504 PLN (1,795 Euros) and 10,082 PLN (2,412 Euros), respectively. **CONCLUSIONS:** UC reduces the quality of life of patients as well as their productivity associated both with absenteeism and presenteeism. The greater the disease activity, the greater the indirect costs and the lower the utility.

PSY61

COMPREHENSIVE EVALUATION OF THE DISEASE BURDEN ASSOCIATED WITH HEMOPHILIA A IN CHINA

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OBJECTIVES: To estimate the disease burden of hemophilia A (HA) in the adult and pediatric populations in China. **METHODS:** A burden of disease model was developed to estimate the total number of bleeds, potential target joints, patients at risk of joint disability, hemorrhage-associated deaths, and days missed from school/work due to HA under the current standard of care in China. A literature review was conducted to inform inputs for the model. The number of target joints, risk of joint disability and hemorrhage-associated deaths were calculated based on their relationship with the number of bleeds. A scenario analysis was carried out to evaluate the clinical burden when the diagnosis/treatment rate improved from 10%/90% to 100%/100% and when treatment standards were optimized (personalized prophylaxis for severe/moderately severe patients; optimized on-demand treatment for mild and moderate patients). **RESULTS:** The model estimated there to be 109,426 people with HA in China, of whom 5,732 and 3,821 were treated adult and pediatric patients, respectively. Amongst treated adults/children, there were 134,191/75,492 bleeds, leading to 8,350/4,694 potential target joints and 27/15 hemorrhage-associated deaths annually under current standard of care. 3,918/2,612 treated adults/children were at risk of disability and 260,562/128,147 days were collectively missed from work/school. In untreated patients, an additional 2.4 million bleeds, 0.15 million target joints and 484 hemorrhage-associated deaths were estimated to occur annually. 57,813 untreated patients were at risk of disability and 5.1 million additional days were estimated to be lost from work/school by these patients. The scenario analysis demonstrated a reduction in the total annual number of bleeds by 95% in the treated and untreated population under optimized diagnosis and treatment standards. **CONCLUSIONS:** HA is a severe health issue in China, associated with burdensome clinical implications and societal costs. The clinical burden may be substantially reduced by improving diagnosis and expanding access to prophylaxis treatment.

PSY62

THE COST OF ILLNESS OF ULTRA-RARE CRYOPYRIN ASSOCIATED PERIODIC SYNDROMES (CAPS) IN ITALY: PRELIMINARY RESULTS FROM THE RADICEA PROJECT

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OBJECTIVES: The ERANET-PRIOMEDCHILD RaDiCEA Project is an international, multicentre, longitudinal observational study evaluating the effectiveness and the economic dimension of the ultra-rare cryopyrin associated periodic syndromes (CAPS) in patients with the indication for either use of anti IL-1 agent or other than anti IL-1; the present study presents results of interim analysis about the cost-of-illness (COI) of 11 patients (6 females, age range: 1-43 years) with CAPS from two Italian centres. **METHODS:** COI analysis was performed considering a bottom-up approach. Resource consumed estimations was based on clinical and economic data collected retrospectively and prospectively by ad-hoc questionnaires administered to patients or their proxy (on direct health and non-health costs, indirect costs) and to the budget and management control officer at each centre to collect information about the healthcare system type, hospitalization reimbursement system, payment for drugs outside the hospital setting, payment of special schools and