Furthermore, the highest approved ICER value of all NICE appraised rare disease submissions was £240,000/QALY for Cystic Fibrosis. The studies considered lifetime horizon and two studies two-five and one-year horizon. Diabetics and non-diabetics patients were taken into account in almost all models. Changes in body mass index (BMI) promoted by the pharmacotherapy were correlated to the risk of experiencing coronary heart disease, diabetes, or death in ten studies. The rare drug models took into account natural BMI increasing due to population ages. Besides BMI, some models were sensitive to changes in total cholesterol, HDL, systolic blood pressure and glycerated hemoglobin for diabetics. Finally, exploratory data from Framingham Heart Study (FHS) were the most common ones used to populate models and competing risks events were not discussed in most of studies. CONCLUSIONS: Health-state transition modelling seems to be the most appropriate methodology to assess orphan therapies. Future studies should explore other co-morbidities and competing risks events. Furthermore, equations used to populate models must be coherent to target population characteristics.

PSY127

ORPHAN AND ULTRA-ORPHAN TECHNOLOGIES: EUROPEAN AND AUSTRALIAN PAYER PERCEPTIONS

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OBJECTIVES: To understand European Union (EU) and Australian payer perceptions and challenges in the evaluation of emerging health technologies with orphan and ultra-orphan designations in France. METHODS: In-depth, qualitative, one-on-one interviews were conducted with payer decision makers representing Australia and the EU-S. France, Germany, Italy, Spain, and the United Kingdom from the RTI Health Solutions Payer Advisory Panel. RESULTS: Payers identify the biggest challenges as lack of evidence of added clinical and economic value for patients, patients, and prescribers to fund the ever-increasing numbers of orphan and ultra-orphan technologies, which are often very expensive and have limited clinical evidence to establish the disease and its burden and unmet needs was consistently highlighted. All payers estimated that spending for orphan and ultra-orphan technologies will increase significantly in the next 5 years, leading to concerns over future funding and budgets. Clawsbcks in France will be common, employment of the cost-benefit and prioritization in Italy, Spain, and the UK are not expected. Payers wanted to see better-defined patient populations and unmet needs accompanied by well-defined treatment courses (e.g., when to stop treatment). Benefits of new technologies will not be captured in traditional health economic analyses, thus increasing uncertainty. Bridging the clinical evidence with other robust data will be critical. The cost to the systems was highlighted as a consistent concern. More emphasis on showing the value, affordability, comparative effectiveness, and cost offsets is critical. CONCLUSIONS: Payers are seeking more value-based information to better inform decision making in the evaluation of new orphan and ultra-orphan technologies. The challenge lies with the value of the new technology and who is judging that value. Rising costs of orphan and ultra-orphan technologies will have more impact on market access in the future, with increasing resistance to high prices.

PSY128

DOES A NEW PHARMACOECONOMIC MODEL IS DEMANDED FOR OBESITY’S PHARMACOTHERAPY ASSESSMENT?

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OBJECTIVES: To describe and discuss existing pharmacoeconomic models assessing obesity’s pharmaco-economic drug treatments with anti-obesity drugs. METHODS: Medline and EMBASE were searched for cost-effectiveness or cost-utility-anslysis reporting in full decision modelling used. It was eligible for assessment only studies which used the EQ-5D and/or the preference-weighted EQ-5D (EuroQol). The Life-Year Gained (LYG) and Cost-Utility Analysis (CUA) were also the outputs considered. Results: 114 articles were identified. Fifteen different models. The final common methodology used was health-state transition modelling followed by life-table. The vast majority of included studies were carried out in Europe, being 4 of them in the UK. Only 2 of the studies considered lifetime horizon and two studies two-five and one-year horizon. Diabetics and non-diabetics patients were taken into account in almost all models. Changes in body mass index (BMI) promoted by the pharmacotherapy were correlated to the risk of experiencing coronary heart disease, diabetes, or death in ten studies. The rare drug models took into account natural BMI increasing due to population ages. Besides BMI, some models were sensitive to changes in total cholesterol, HDL, systolic blood pressure and glycerated hemoglobin for diabetics. Finally, exploratory data from Framingham Heart Study (FHS) were the most common ones used to populate models and competing risks events were not discussed in most of studies. CONCLUSIONS: Health-state transition modelling seems to be the most appropriate methodology to assess obesity therapies. Future studies should explore other co-morbidities and competing risks events. Furthermore, equations used to populate models must be coherent to target population characteristics.

PSY112

EPIDEMIOLOGY AND HEALTHCARE SERVICES UTILIZATION FOR RARE DISEASES IN ITALY

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OBJECTIVES: Rare diseases (RD) are an important bench test case for healthcare services organization. This for both moral and ethical aspects which implies knowing how to cope with the needs of a vulnerable sector of population. At the same time RD its a challenge for healthcare service organizations, which are called to respond to individual requirements. However, both RD patients and informal caregivers quality of life has been recognized, including hematologi-

PSY130

COMPREHENSIVE REVIEW OF ORPHAN DRUGS POST-AMNOG IN GERMANY

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OBJECTIVES: In Germany, orphan drugs are assessed based on their EMA orphan designation and expected annual budget impact. Per German law, the additional benefit for the product is assessed under the orphan drug dossier. Products with expected annual budget impact of ≤50 million annually qualify and therefore are only required to submit an abbreviated dossier. Products with expected budget impact >50 million must submit a full dossier and are assessed for the ability to demonstrate benefit. Up to now, 145 orphan drugs have been reviewed post-AMNOG in Germany, including their resulting price rebates. METHODS: GfK reviewed all EMA orphan-designated market authorizations since 2011 and cross-referenced these with the dossier submitted. GfK then reviewed G-BA resolutions to determine which target populations, population sub-populations, 8 (50%) products achieved ‘minor additional benefit’, 6 (37.5%) products were assessed as orphan drugs in Germany. Of these, 10 were indicated for at least two chronic conditions (€4,500.2). In terms of gender the figure for women (3,359.3) is significantly lower than that for males (€6,856.6). The total impact on R&D is €223.9 M, equal to 1.21% of regional spending. Patients with rare ultra-rare diseases are characterized by costs (€6,953.6 pro capita) significantly above the average. CONCLUSIONS: the research give some original insight on the prevalence and economic impact of RD patients in a large population. It permits to appreciate the potential impact of RD drugs for patients with such an ultra-rare condition, as well as financial risk for the local health care units due to prevalence concentration.

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