efficiency, resulting in trials becoming smaller and less costly. However, the use of composite end points is biased against drug approval programs, as two treatments that are equal on individual components are not considered equivalent if their outcomes are treated as equal. We aimed to examine patients’ perspectives regarding the use of composite end points and the utility patients put on possible unfavourable outcomes of treatment.

METHODS: In this single-centre, prospective study, 160 patients who had undergone PCI were interviewed. The first part of the study involved a questionnaire that was distributed to patients who had undergone PCI within a year post-intervention (CAD) at the Thoraxcenter Twente, Medisch Spectrum Twente (Enschede, The Netherlands) between January and June 2016, were invited to participate in this study. A total of 126 (90%) patients gave consent to participate in this study; A novel methodology, a survey-based BWs choice experiment, was conducted to determine the relative importance of each component end point to CAD-patients. RESULTS: Patients were asked to evaluate PCI within a year post-intervention (OR=2.3, p<0.001). The MI where symptoms disappear within three months (OR=2.3, p<0.001) and the angina pectoris who has symptoms within 6 months (OR=0.1, p<0.001). The subgroup (revascularization procedure, prior MI, and prior revascularization) differences can be found for the relative weights attributed to “death” versus “stroke causing permanent disability”. CONCLUSIONS: CAD-patients do not consider the components of a composite endpoint equally. The fact that patients do weight the individual components differentially has significant implications for trial statistics, and the interpretations of trial data, since these can be misleading.

PRING POLICY STUDIES

PR1 ASSOCIATION BETWEEN THE PRICES OF ORPHAN DRUGS IN ONCOLOGY AND THE PATIENT POPULATION SIZES

Jarosław K. Tomula
Aix-Marseille University, Marseille, France

OBJECTIVES: High prices of orphan drugs are often linked to the necessity to recover the development costs from sales to small patient populations. If this is true, drugs with smaller target population sizes should be more costly. We sought to compare the prices of orphan drugs in oncology with their respective patient population sizes. METHODS: A list of orphan drugs designated by the FDA between June 2011 and June 2016 was retrieved from the FDA website. We included: a) drugs approved by the FDA; b) treatments in oncology. We exclude: a) products not specifically indicated to treat cancer (e.g., imaging, palliative care or treatment of a cancer-associated condition). Diseases prevalence data was obtained from the FDA orphan drug approval reviews. Average Wholesale Prices per unit were obtained from the Micromedex database. Prices per year of treatment were calculated based on the drugs’ dosage from the FDA labels. We used descriptive statistics to compare drug prices per year of treatment to the target populations of patients for each indication. RESULTS: Out of 187 orphan designated indications, 70 led to drug approvals by the FDA. 37 approvals were in oncology. Eighty-two had two approved indications and one had five indications. There were 25 unique regimes of which two were for drug combinations. There was no clear association between the drug prices and the sizes of their respective patient populations. CONCLUSIONS: The current orphan drug policies have encouraged the development of novel treatments, but have also led to extremely high prices of these drugs. Whereas drug prices may depend on factors other than population size alone, our findings suggest that there is no apparent link between the prices and the sizes of their respective patient populations. This should lead to revisiting orphan drug policies that encourage innovation, but that are based on drivers other than potential market size alone.

PR2 PAY FOR PERFORMANCE: A PROPOSAL FOR AND SIMULATION OF REAL-TIME OUTCOMES-BASED PHARMACEUTICAL PRICING USING ROUTINELY COLLECTED DATA

Burt T1, Lee A2, Tufail A3
1University College London, London, London, UK 2University of Washington, Seattle, WA, USA 3Moorfields Eye Hospital NHS Foundation Trust, London, UK

OBJECTIVES: Electronic medical records (EMR) provide a rich source of routinely collected real-world data on health outcomes and resource use, which could be potentially used in real-time decision making to adjust pharmaceutical pricing for each individual patient in order to reflect the value a product delivers to the health care system. Our aim was to simulate how real time outcomes-based pricing could be operationalised in the UK. Simulation using Roux was to simulate how real time outcomes-based pricing could be operationalised in the UK. Simulation included data on 8,681 patients treated across 1,416 days. At the end of the simulation, the mean number of QALYs accumulated by patients was 3.05 ± 0.25. The model was calibrated to 2.56 in the natural history arm over the five year time horizon. CONCLUSIONS: Real world data may be used to monitor the cost-effectiveness of on-market drugs and to regularly refine their prices based on the value delivered in clinical practice. Performance-based pricing could be used to negotiate earlier market access for pharmaceuticals in advance of mature data on cost-effectiveness.

PR3 DIFFERENCES IN PRICING POLICIES FOR GENERIC AND BIOSIMILAR MEDICINES

Vogel A1, Schneider R, Gomboa M, Zimmermann N
1Fries Dauphine University, Paris, France 2Université Paris-Dauphine, Paris, France

OBJECTIVES: The variation in health system regimes across different countries has been a key driver of the pharmaceutical industry in the last year time horizon. The objectives of this study are in threefold: First to the assess trends in prices of anti-cancer drugs approved by the FDA; b) treatments in oncology. We exclude: a) products not specifically indicated to treat cancer (e.g., imaging, palliative care or treatment of a cancer-associated condition). Diseases prevalence data was obtained from the FDA orphan drug approval reviews. Average Wholesale Prices per unit were obtained from the Micromedex database. Prices per year of treatment were calculated based on the drugs’ dosage from the FDA labels. We used descriptive statistics to compare drug prices per year of treatment to the target populations of patients for each indication. RESULTS: Out of 187 orphan designated indications, 70 led to drug approvals by the FDA. 37 approvals were in oncology. Eighty-two had two approved indications and one had five indications. There were 25 unique regimes of which two were for drug combinations. There was no clear association between the drug prices and the sizes of their respective patient populations. CONCLUSIONS: The current orphan drug policies have encouraged the development of novel treatments, but have also led to extremely high prices of these drugs. Whereas drug prices may depend on factors other than population size alone, our findings suggest that there is no apparent link between the prices and the sizes of their respective patient populations. This should lead to revisiting orphan drug policies that encourage innovation, but that are based on drivers other than potential market size alone.

BREAKOUTS SESSION IV

MEDICATION ADHERENCE STUDIES

AD1 REAL-WORLD IMPACT OF GENDER, AGE AND SOCIO-ECONOMIC STATUS ON TYPE-2 DIABETES MELLITUS (T2DM) PATIENTS DISEASE ENGAGEMENT AND ADHERENCE WITH TREATMENT

Higgins V, Leith A, Renford M, Siddall J, Penny R
Adelphi Real World, Bollinghurst, UK

OBJECTIVES: It is hypothesised that gender, age and socio-economic status (SES) shapes adherence in engagement with disease, life-style choices and adherence. This analysis investigated the impact of these non-modifiable factors amongst T2DM patients. METHODS: Data were drawn from the 2015 Adelphi Diabetes Disease Specific Programme (DSP) in T2DM across SEU/USA. The DSP is a real-world, cross-sectional survey involving diabetes specialists, primary care physicians (PCPs), and